Q4 2021 Results
Investor presentation

NOVARTIS | Reimagining Medicine

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Chief Executive Officer

Company overview

Novartis presents an attractive profile for investors

1
Clear strategy

Delivering on strategy as a focused medicines company, powered by technology platforms

2
Attractive growth profile

Confident in **4%+** sales CAGR (2020 to 2026), above peer median growth beyond 2026

High 30s IM margin

3
Strong mid/late-stage portfolio

Breadth and depth, **20+** assets with USD ≥1bn potential, fuel further growth to 2030 and beyond

4
Platform leadership

Continue to develop leadership across TPD, Cell, Gene, RLT, xRNA platforms

5
Balanced capital allocation

Aims to combine investing in core business and returning excess capital to shareholders

TPD – Targeted Protein Degradation RLT – Radioligand Therapy

Our strategy

Focused medicines company powered by technology leadership in R&D, world-class commercialization, global access and data science

Where to play | our focus

Strengthen our core therapeutic areas

Advance our leading technology platforms

Accelerate our 4 priority geographies

Transform Sandoz

How to win | our five priorities

Embrace operational excellence every day

Unleash the power of our people

Deliver transformative innovation

Go big on data and digital

Build trust with society

Our aspiration

Innovation power
Top 3 innovator

Returns
High 30s IM margin, attractive ROIC[1]

Growth
Consistent above peer median average growth

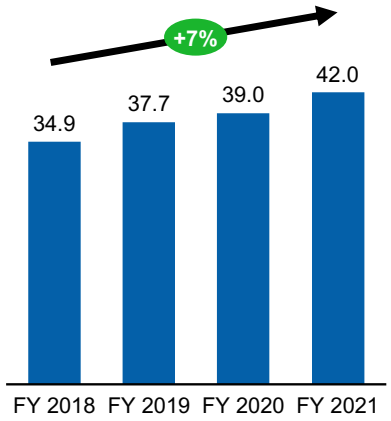
ESG
Global leader in material ESG factors

1. Return on invested capital

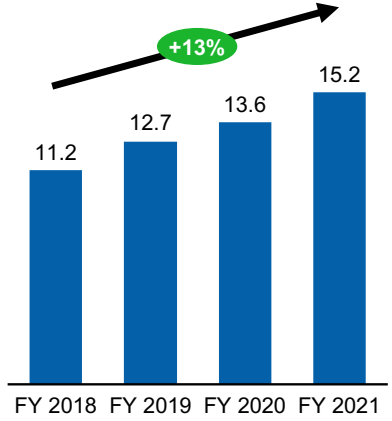
Continuing our track record of consistent top-line growth, margin expansion, strong FCF

Consistent strong operating performance driven by IM

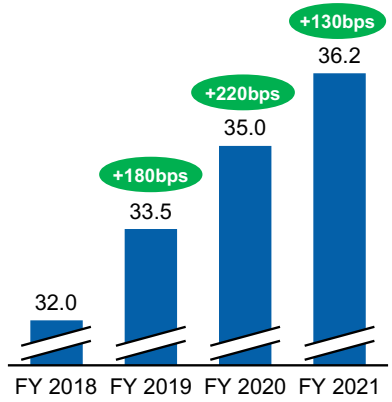
IM Sales
USD bn, % CAGR cc



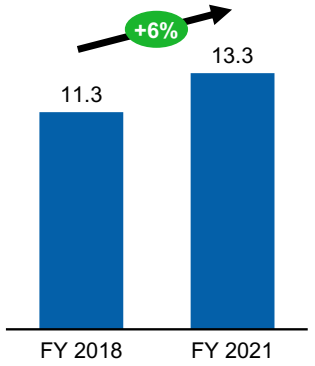
IM Core OpInc
USD bn, % CAGR cc



IM Core Margin
(%), growth bps cc



Group FCF
Cont. Ops, USD bn, % CAGR USD

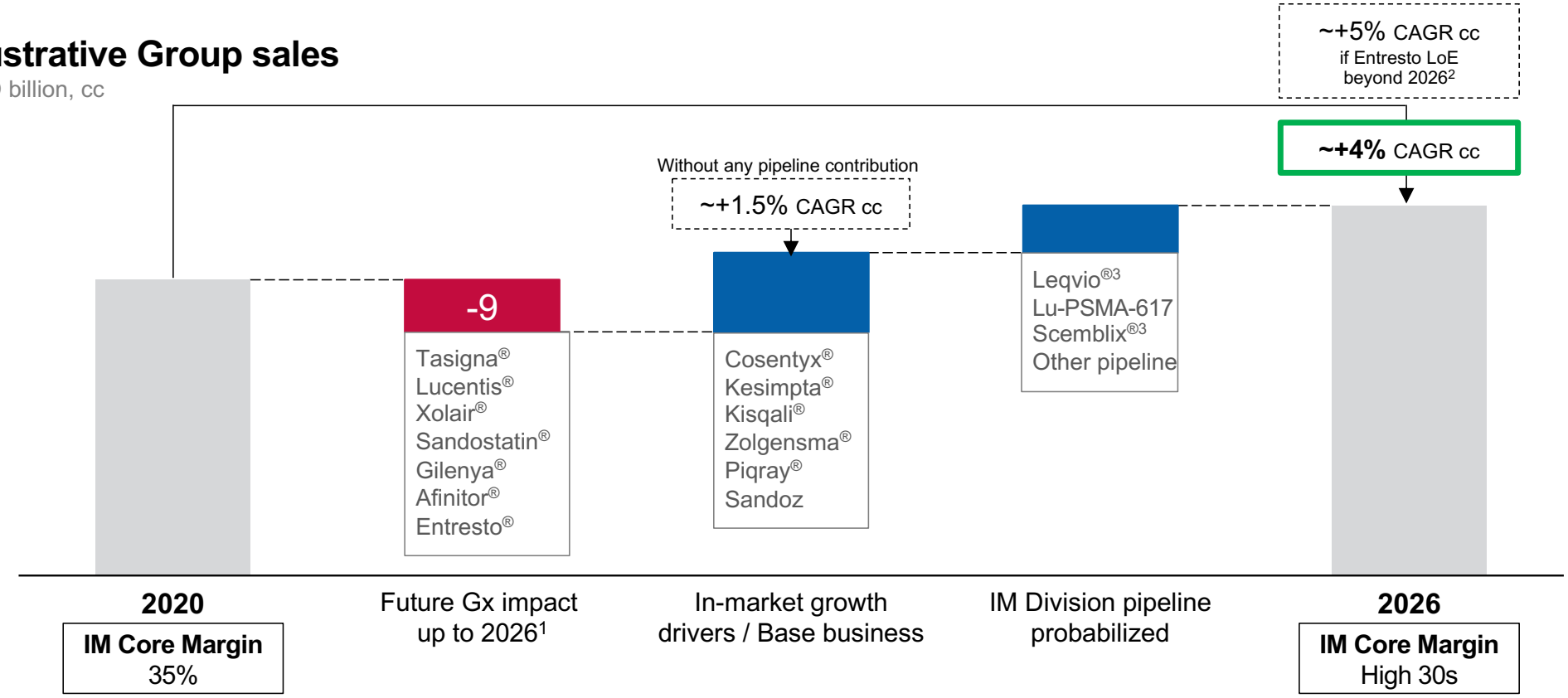


IM – Innovative Medicines

Expect to grow sales 4%+ CAGR 2020 - 2026

Illustrative Group sales

USD billion, cc



Excludes potential impact from US healthcare reform. Compared to R&D Day 2021, removed Ligelizumab in CSU. 1. Estimated based on relevant patents; further extensions possible. Additional products include Promacta, Q-Family and Votrient. 2. For internal forecasting purposes we do not expect Gx in US at least until 2025. 3. Approved in US.

Delivered strong Q4 performance across our value drivers

Growth[1] 1

Q4 Group sales **+6%**; FY +4%

Q4 IM sales **+7%**; FY +6%

Q4 Sandoz sales **+2%**; FY -2%

Innovation 3

Leqvio® approved in US

Cosentyx® Ph3 studies met primary endpoint in HS

Ianalumab positive Ph2 in Sjögren's

T-Charge™ platform positive data in DLBCL and MM

Business development (ociperlimab, Gyroscope, ensovibep, UCB0599)

Productivity[1] 2

Q4 Group core operating income **+12%**; FY +6%

Q4 IM core operating income **+15%**; FY +10%

Q4 IM core margin 33.6% (**+2.4%**pts cc); FY 36.2%

ESG 4

Improved scores for **MSCI**, ATMI AMR Benchmark, S&P Global

Environmental targets on track (-34% Scope 1,2 GHG, -56% waste)

Refreshed commitment statement on **human rights**

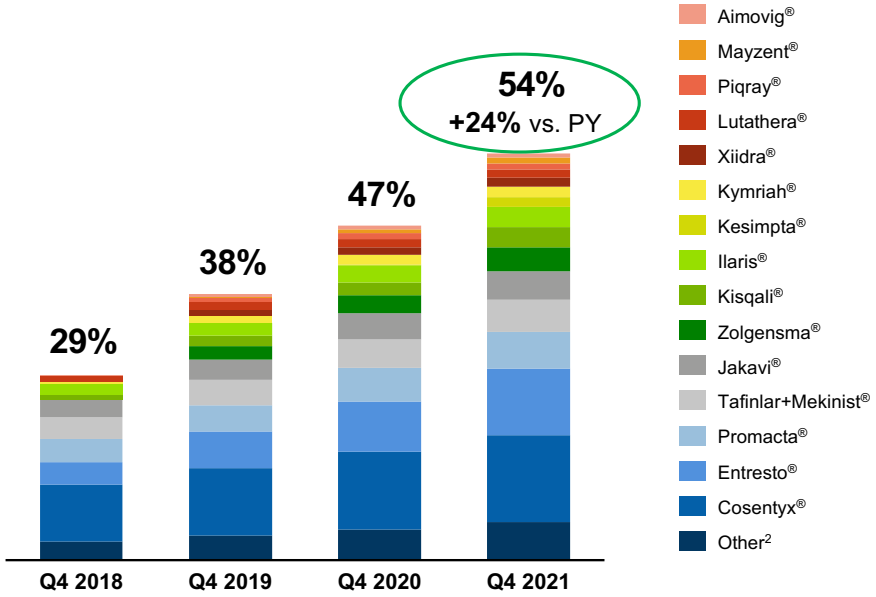
IM – Innovative Medicines division HS – Hidradenitis suppurativa DLBCL – Diffuse large B cell lymphoma MM – Multiple myeloma 1. Q4 sales growth for Group, IM and Sandoz includes +1% point impact from a reclassification of contract manufacturing from other revenue to sales. Sandoz FY sales growth also benefited +1% point from this reclassification. Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 49 of Condensed Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Key growth drivers grew +24% in Q4, representing 54% of IM sales

Q4 sales[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto	949	233	34%
Cosentyx	1,243	134	13%
Kesimpta	147	133	nm
KISQALI	285	101	58%
zolgensma	342	88	36%
Tafinlar + Mekinist	458	50	14%
PROMACTA	518	47	12%
ILARIS	284	44	23%
Xolair	373	38	15%
JAKAVI	408	32	12%
xiidra	134	26	24%
MAYZENT	81	24	46%

Key growth drivers 54% of IM sales, growing 24% in Q4



nm – not meaningful 1. Innovative Medicines division. 2. Includes Xolair®, Beovu®, Adakveo®, Tabrecta®, Luxturna®, Enerzair®, Atectura® , Scemblix® and Leqvio®. Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 49 of Condensed Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Double digit FY growth for key brands

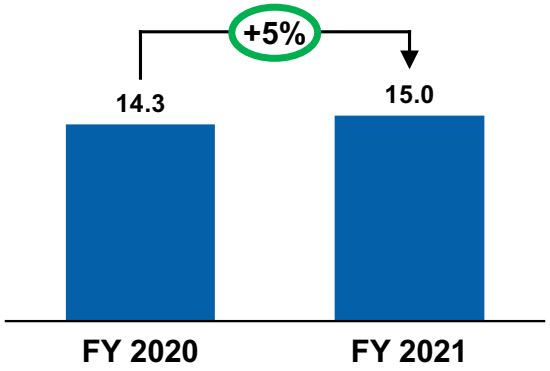
Cosentyx®	Entresto®	zolgensma®	KISQALI®	Kesimpta®	LEQVIO®
USD 4.7 bn	USD 3.5 bn	USD 1.4 bn	USD 0.9 bn	USD 0.4 bn	nm
+17%	+40%	+46%	+36%	nm	nm
Est. CAGR (2020-26) Low double digit	Est. CAGR (2020-26) Double digit until LoE	Est. CAGR (2020-26) Low to mid teens	Est. CAGR (2020-26) Low 30s[2]	Est. CAGR (2020-26) nm	Est. CAGR (2020-26) nm
Peak sales **USD >7bn**	Peak sales **USD >5bn**	Peak sales **multi-bn**[1]	Peak sales **multi-bn**	Peak sales **multi-bn**	Peak sales **multi-bn**
US LoE 2029+	US LoE 2025-2036	US LoE 2031+	US LoE 2031+	US LoE 2031+	US LoE 2036+

nm – Not meaningful. All growth rates in constant currencies (cc). US LoEs are estimated based on relevant patents; further extensions possible. 1. Including Zolgensma IT. 2. Including Kisqali adjuvant.

IM performing well across geographies in 2021

US

USD bn, % cc

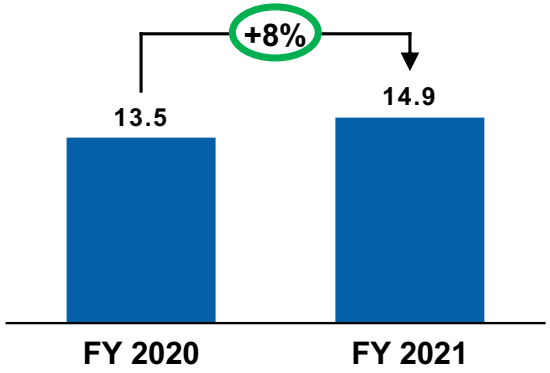


Key growth drivers

Cosentyx®, Entresto®, Kesimpta®

Europe

USD bn, % cc

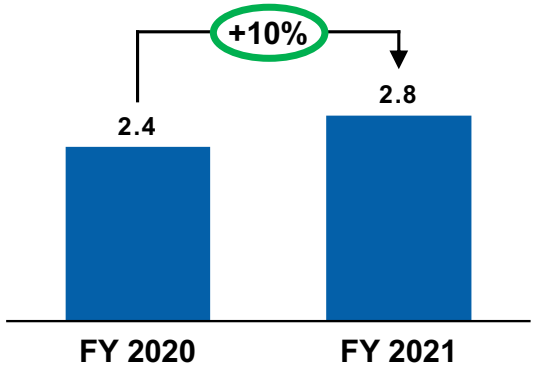


Key growth drivers

Entresto®, Zolgensma®, Kisqali®, Jakavi®

China

USD bn, % cc



Key growth drivers

Cosentyx®, Entresto®, Lucentis®

Broad pipeline of novel medicines continued to progress in Q4

● Negative ● Positive ● Mixed results

Approvals

SCEMBLIX®	US: CML 3L
Cosentyx®	US: JPsA & ERA
LEQVIO®	US: Hyperlipidemia
Kesimpta®	CN: rMS

Submissions

Alpelisib	US: PROS
^{177}Lu-PSMA-617	EU: mCRPC, post-taxane
KYMRIAH® (tisagenlecleucel) Suspension for IV infusion	JP: r/r Follicular lymphoma

Designations

Branaplam	FDA Fast Track designation in Huntington's disease
Alpelisib	FDA Priority Review in PROS

Readouts and publications

●	**Cosentyx®**	Ph3 – HS (SUNSHINE and SUNRISE)
●	**Ligelizumab**	Ph3 – CSU (PEARL 1 and 2)[2]
●	**Pelacarsen**	Prevalence – Lp(a) (HERITAGE)
●	**Ianalumab**	Ph2b – Sjögren's
●	**Cosentyx®**	Ph2 – PsA IV (INVIGORATE-2)
●	**Ensovibep**	Ph2 – COVID-19 (EMPATHY)
●	**YTB323**	Ph1 – DLBCL
●	**PHE885**	Ph1 – Multiple myeloma

Major Phase 3 study starts

Remibrutinib	MS (REMODEL-1/-2); CSU (REMIX-1/-2)
Ligelizumab	Food allergy (peanut[1]); CINDU (PEARL-PROVOKE)

Selected milestones 1. NCT04984876. 2. Superiority demonstrated vs. placebo but not vs. omalizumab. See last slide for all abbreviations.

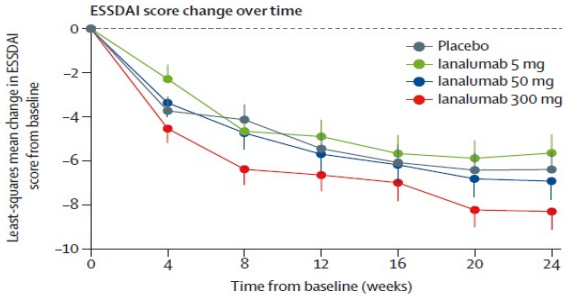
Important readouts for ianalumab, Cosentyx® and ligelizumab

Selected data readouts

Ianalumab

Ph2b Sjögren's Syndrome

- High unmet need, 400k patients[1], no DMT
- Primary efficacy endpoint met; dose response defined as change in ESSDAI from baseline at 24 weeks[2]



- Good tolerability

Next steps

- Ph3 start H2 2022

Other indications: Lupus Nephritis (Ph3 to be initiated 2022), SLE and AIH (Ph2), B-cell malignancies (Ph1/2)

Cosentyx®

Ph3 HS (SUNRISE/SUNSHINE)

- High unmet need in HS[3]
- Primary efficacy endpoint of HiSCR at week 16 met in both studies
- HiSCR response: **≥50%** decrease[4]
- Favorable safety profile confirmed

Next steps

- Studies remain blinded, data will be presented after week 52
- Proceeding to 1st submission Q2

Ligelizumab

Ph3 CSU (PEARL1&2)

- Superiority demonstrated vs. placebo but not vs. omalizumab

Next steps

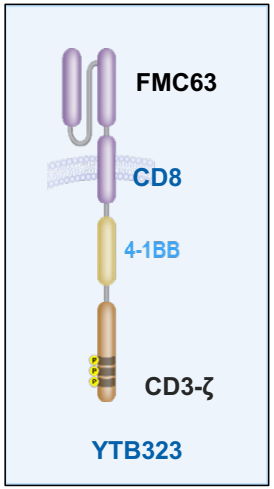
- Evaluation of Ph3 data continuing
- Data release on completion H2 2022
- CINDU, food allergy studies continue

HiSCR: Hidradenitis Suppurativa Clinical response 1. with moderate to severe SjS disease in G7. 2. Bowman et al, The Lancet 2021, in press. 3. Available treatments do not adequately reduce disease activity or prevent disease progression; ~400k patients with moderate to severe HS: 200k patients US, 200k patients EU5; source: British Journal of Derm. 4. in Abscess and Inflammatory Nodule count with no increase in the no. of draining fistulae.

First data presented for two lead constructs on T-Charge™

Designed to provide fast access to therapy, increased rates of response and longer durability

YTB323 is an autologous CD19-directed CAR-T cell therapy in Ph1 for DLBCL and ALL

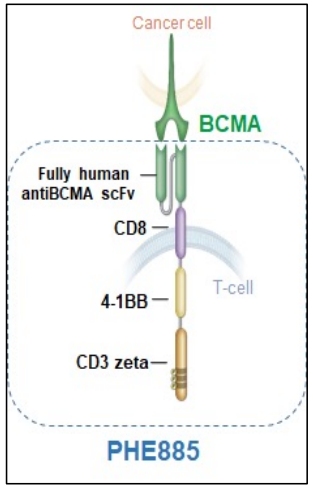


- DLBCL is the most common type of NHL, ~31% of NHL in Western countries
- Promising initial efficacy results: **73% CR** rate at month three in patients with DLBCL (n=16)
- Preliminary safety profile similar to Kymriah in JULIET study

Next steps

- Ph3 trial in DLBCL to start in 2022

PHE885 is an autologous BCMA-directed CAR-T cell therapy in Ph1 for MM



- Multiple Myeloma (MM) comprises ~10% of hematologic malignancies
- Encouraging preliminary data: **100% ORR** in r/r MM patients (n=6)

Next steps

- Dose-finding Ph1 study is ongoing
- Ph2 initiation in 2022

T-Charge™ platform preserves T-cell stemness: May lead to deep and durable responses, improved long-term outcomes, better safety
Rapid manufacturing, lower cost of goods and increased scale

DLBCL – Diffuse large B cell lymphoma NHL – Non-Hodgkin Lymphoma

Business development activities adding 4 new mid/late stage high potential medicines

GYROSCOPE VISION FOR LIFE

Acquisition

GT005: One-time subretinal Ph2 gene therapy that could transform care for geographic atrophy, a leading cause of blindness[1]

BeiGene

Option

Ociperlimab: Ph3 TIGIT inhibitor with the potential to treat a wide range of solid tumors; development program includes 6 global trials in lung cancer, ESCC, cervical cancer

MOLECULAR partners

Opt-in[2]

Ensovibep: Multi-specific Ph2 DARPin®, specifically designed to block the receptor binding domains of SARS-CoV-2 spike protein

ucb

Co-development / co-commercialization

DLX313 (UCB0599): Potential first-in-class, small molecule, alpha-synuclein misfolding inhibitor in Ph2 Parkinson's Disease

DARPin – Designed Ankyrin Repeat Protein. 1. Completion of the transaction is subject to customary closing conditions. Novartis and Gyroscope will continue to operate as separate and independent companies until closing. 2. License Agreement with Molecular Partners signed.

Confident in future growth driven by our strength and depth in cardio-renal, immunology, neuroscience...

Selected assets, nearly all with exclusivity into 2030+

New for Q4

Cardio-Renal

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Leqvio®	Hyperlipidemia	●●●	Approved	-
	CVRR-LDLC		Ph3 ORION-4 and VICTORION-2-PREVENT ongoing	2026+
Iptacopan[1]	IgAN	●●●	Ph3 APPLAUSE-IgAN ongoing	2023[2]
	C3G		Ph3 APPEAR-C3G ongoing	2023
	iMN		Ph2b ongoing	2026+
Pelacarsen	CVRR-Lp(a)	●●●	Ph3 Lp(a)HORIZON ongoing	2025

Neuroscience

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Zolgensma®	SMA IT	●●●	Ph3 STEER initiating	2025
Branaplam	Huntington's disease	●●●	Ph2b VIBRANT-HD ongoing	2026+
Remibrutinib[1]	Multiple sclerosis	●●●	Ph3 REMODEL-1 and -2 ongoing	2025
DLX313[4]	Parkinson's disease	●●●	Ph2 ongoing	2026+

Unprobabilized peak sales (USD): ● <1bn ●● 1-2bn ●●● >2bn

Immunology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Cosentyx®	HS	●●●	Ph3 SUNRISE, SUNSHINE positive readout	2022
	GCA		Ph3 ongoing	2024
	jPsA/ERA		Aproved (US) in Q4	-
	Lupus Nephritis		Ph3 SELUNE ongoing	2026+
	Lichen Planus		Ph2b PRELUDE readout in 2022	2025
Ligelizumab	CSU	●●●	Ph3 PEARL 1, 2 readout	TBC
	Food allergy[3]		Ph3 ongoing	2025
	CINDU		Ph3 PEARL-PROVOKE ongoing	2025
Remibrutinib[1]	CSU	●●●	Ph3 REMIX-1 and -2 ongoing	2024
	Other indications being explored			
Ianalumab	Sjögren's	●●●	Ph3 start in 2022	2026+
	SLE		Ph2a ongoing	2026+
	Autoimmune hepatitis		Ph2b ongoing	2026+
	Lupus Nephritis		Ph3 start in 2022	2026+
Iscalimab	Liver Tx	●●	Ph2b ongoing	2026+
	Sjögren's		Ph2b ongoing	2026+
	HS		Ph2a ongoing	2026+

'Wild Cards' LNA043 (osteoarthritis: Ph2 ongoing), CSJ117 (asthma: Ph2b ongoing, COPD: Ph2 ongoing), QBW251 (COPD: Ph2b readout H1 2022), SAF312 (COSP: Ph2b ongoing), UNR844 (presbyopia: Ph2b readout H2 2022)

1. Peak sales potential based on all studied indications. 2. Based on 9 months UPCR readout (US accelerated approval). 3. Food Allergy indication falls within the Respiratory & Allergy therapeutic area 4. =UCB0599

… and strength and depth in oncology

Selected assets, nearly all with exclusivity into 2030+

New for Q4

Solid Tumors

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Kisqali®	HR+/HER2- BC (adj)	●●●	Ph3 NATALEE readout event-driven, expected end 2022[1]	2023
Canakinumab	NSCLC adjuvant	●●	Ph3 CANOPY-A readout in 2022	2023
Lu-PSMA-617	mCRPC post-taxane	●●●	In registration	-
	mCRPC pre-taxane		Ph3 PSMAfore readout event-driven, end 2022[1]	2023
	mHSPC		Ph3 PSMAddition ongoing	2024
JDQ443 KRAS inhibitor	2/3L NSCLC (mono)	●●●	Ph3 start in H2 2022	2024
	NSCLC (combo)		Ph2 ongoing	2026+
TNO155 SHP2 inhibitor	Solid tumors: multiple combinations being explored in ongoing trials			
Tislelizumab[2]	2L esophageal cancer	●●	In registration	-
	NSCLC		H1 2022 EU submission, H2 2022 2L US submission	2022
	Other indications		Ongoing trials	-
Ociperlimab[2] TIGIT mab	NSCLC	●●●	Ph3 ongoing[4]	
	Other indications		Ongoing trials[4]; additional Ph3 study initiation H2 2022	

Hematology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Scemblix® (asciminib)	CML 3L	●●●	US approved	-
	CML 1L		Ph3 ongoing	2025
Iptacopan[2]	PNH	●●●	Readout in 2022 (APPLY-PNH)	2023
	aHUS		Ph3 ongoing	2025
Sabatolimab	HR-MDS	●●●	Ph2 STIMULUS-MDS-1 continues to PFS readout[3]	2022/2023
			Ph3 STIMULUS-MDS-2 ongoing	
	AML		Ph2 STIMULUS-AML-1 ongoing	2024
YTB323 CD19 CAR-T	Non-Hodgkin's Lymphoma	●●●	Ph3 start 2022	2024
PHE885 BCMA CART-T	Multiple myeloma	●	Ph2 start 2022	2024

Unprobabilized peak sales (USD): ● <1bn ●● 1-2bn ●●● >2bn

'Wild Cards' | NIS793 (mPDAC: Ph3 ongoing, colorectal cancer: Ph2 ongoing)

1. Could move to early 2023. 2. Peak sales potential based on all studied indications; Novartis territories. 3. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial. 4. Active trials are being conducted by BeiGene, option deal.

2022 events[1] (expected)

NME Lead

Regulatory decisions	H1	^{177}Lu-PSMA-617 mCRPC (US/EU)
	H1	alpelisib PROS (US)
	H2	Scemblix® 3L CML (JP/EU)
	H2	tislelizumab ESCC 2L (US)
	H1/H2	Jakavi® acute & chronic GVHD (EU/JP)
	H1/H2	Kymriah® r/r follicular lymphoma (US/EU/JP)
	H1/H2	Beovu® DME (US/EU/JP)
Submissions	H1	ensovibep COVID-19 (US)
	H1/H2	Cosentyx® HS (EU/US)
	H1/H2	tislelizumab NSCLC (EU/US)
	H2	tislelizumab 1L Nasopharyngeal cancer (US)
	H2	Cosentyx® Psoriatic Arthritis IV (US)
Submissions-enabling readouts	H2	canakinumab NSCLC Ph3 Canopy A
	H2	iptacopan PNH Ph3 APPLY-PNH
	H2	Kisqali® HR+/HER2- BC (adj)[2] Ph3 NATALEE
	H2	^{177}Lu-PSMA-617 MCRPC[1], pre-taxane Ph3 PSMAfore

Other readouts	H1	sabatolimab HR-MDS Ph2
	H1	Cosentyx® Lichen planus Ph2 PRELUDE
	H1	Cosentyx® AS IV Ph3 INVIGORATE-1
	H1	icenticaftor COPD Ph2b
	H2	UNR844 presbyopia Ph2 READER
Ph3/pivotal study starts	H1	Cosentyx® peripheral SpA
	H1	OAV101 SMA IT STEER
	H1	ensovibep COVID-19 (EMPATHY Part B)
	H2	JDQ443 NSCLC mono
	H2	ianalumab Sjögren's Syndrome
	H2	ianalumab Lupus Nephritis
	H2	ociperlimab solid tumors
	H2	^{177}Lu-PSMA-617 nmCRPC
	H2	YTB323 2L DLBCL
	H2	OAV101 SMA IT Ph3b STRENGTH

1. Selected. 2. Event driven, could move to early 2023.

Sandoz stabilizing in Q4; Biosimilars expected to drive future growth

Sandoz stabilizing

Q4 sales USD 2.5bn (+2% cc)[1]
FY sales USD 9.6bn (-2% cc)[1]
FY Biopharma sales USD 2.1bn (+7% cc)

Outlook 2022: Sales broadly in line with PY

Assumptions

- Cough & Cold revert to pre-COVID levels
- Bio continues to outperform where we compete
- Continued gross margin headwinds due to price erosion and unfavorable mix

Biosimilar launches expected to drive material growth from 2024

Significant LOE opportunity

Targeting USD 80bn originator sales (2030)

Critical success factors

- ☑ Leading pipeline: 15+ assets
- ☑ Manufacturing scale and expertise
- ☑ Development and regulatory experience
- ☑ Global footprint
- ☑ Experience in commercialization
  Leading in Europe; expanding US, RoW

Strategic review of Sandoz is progressing, expected to provide an update, at latest, by the end of 2022

1. Q4 and FY sales growth for Sandoz includes +1% point impact from a reclassification of contract manufacturing from other revenue to sales.

We remain disciplined and shareholder-focused in our capital allocation

Investing in the business

Investments in organic business

USD 9bn R&D[1]
USD 1.4bn capital investments

Value-creating bolt-ons

USD 30bn (approx.) since 2018[2]

Returning to shareholders

Growing annual dividend in CHF

USD 7.4bn paid out in 2021; proposed DPS increase **+3% CHF; +6% USD**

Share buybacks

USD 2.8bn executed in 2021
USD 15bn (up to; by end 2023)[3]

1. Core R&D actuals 2021. 2. Until Q4 2021. 3. Announced on December 16, 2021.

Selected ESG highlights from Q4

- **Novartis Biome Sub-Saharan Africa (SSA):** Developing innovative / technology-driven solutions in SSA
- **Refreshed human rights commitment statement:** Focusing on 12 human rights areas[1]
- **Disability inclusion:** Joined The Valuable 500, supporting a global movement to drive systemic change
- **Environmental targets on track:** -34% Scope 1,2 GHG emissions excluding offsets[2]; -56% waste disposal (2025 target: reduce waste disposal by half); engaging top suppliers on 'Green Expectations'
- **Improved scores** for MSCI Controversy, ATMI AMR Benchmark, S&P Global ESG rating[3] for 2021

1. In line with United Nations Guiding Principles for Human Rights. 2. 2021 GHG Scope 3 will be published in H1 2022. 3. Included in the DJSI World, DJSI Europe

Top 2022 priorities for Novartis

1. **Successful launches:** Leqvio®, Kesimpta®, ^{177}Lu-PSMA-617, Scemblix®
2. **Maintain growth momentum:** 
3. **Progress pipeline**: 20+ assets with significant sales potential, approval by 2026, on track
4. **Optimize portfolio:** Sandoz review, update end 2022; disciplined BD
5. **Deliver returns:** Continue productivity initiatives, especially manufacturing, business services
6. **Reinforce foundations**: Culture to drive performance, data science to drive value, ESG leadership

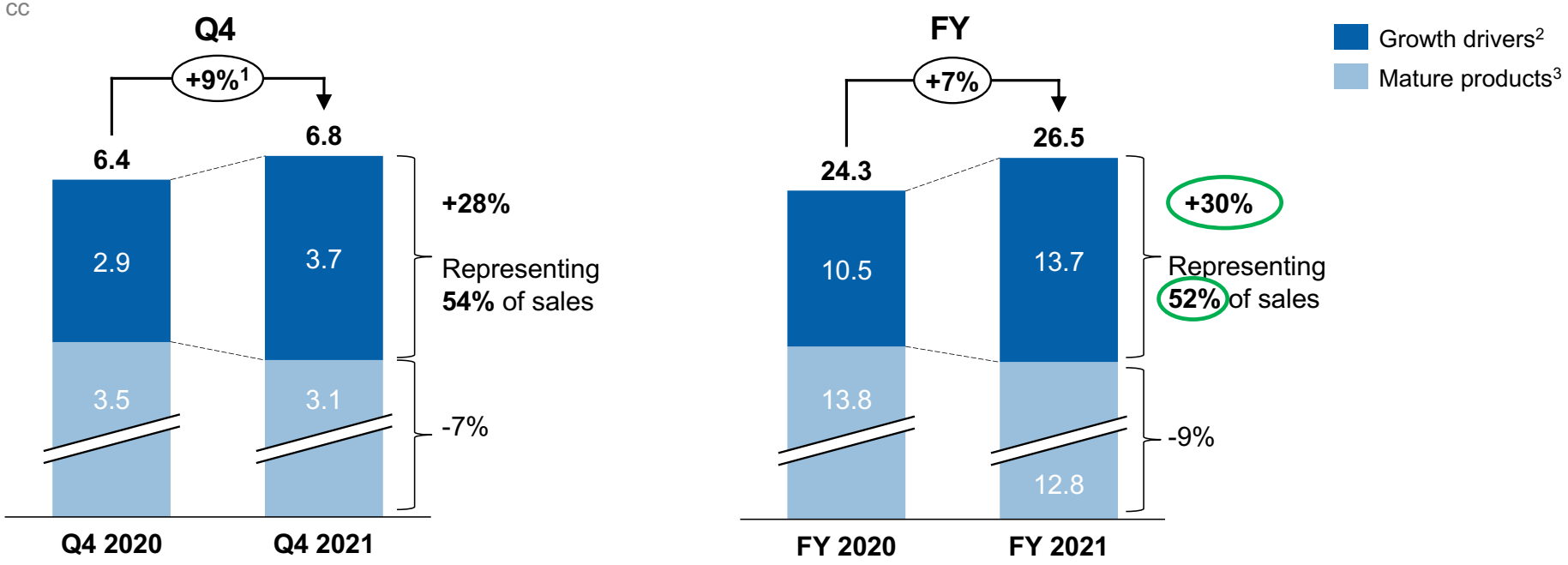
Marie-France Tschudin

President, Novartis Pharmaceuticals

FY Pharmaceuticals sales grew +7%, growth drivers represent 52%

Pharmaceuticals net sales

USD bn, % cc

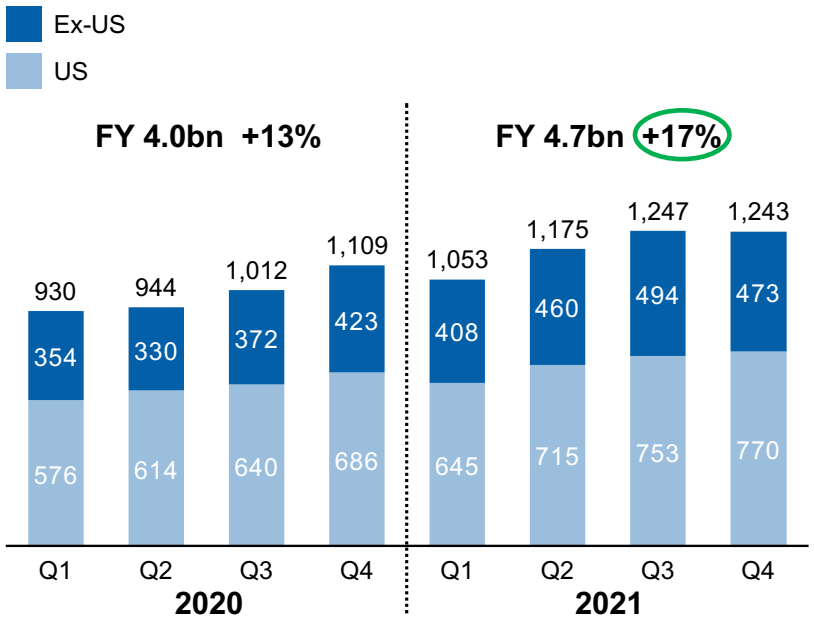


All % growth relate to cc unless otherwise stated. 1. Q4 sales growth for Pharmaceuticals includes +2% points impact from a reclassification of contract manufacturing from other revenue to sales. 2. Zolgensma®, Kesimpta®, Mayzent®, Beovu®, Luxturna®, Leqvio®, Enerzair® and Atectura®, Cosentyx®, Entresto®, Xolair® , Ilaris® , Xiidra® and Aimovig®. 3. All other brands.

Cosentyx® maintains strong market position; progressing LCM

Sales evolution

USD m, % cc



Maintaining strong growth and market position

- Demand driven across indications in US, Europe, China

Expanding clinical differentiation

- Hidradenitis suppurativa Ph3 positive
- Approved for JPsA and ERA in the US
- Approval for PsO flexible dosing in EU
- GCA Ph2b positive; Ph3 ongoing
- Anticipate 10+ indications overall

Expect double-digit growth in 2022

- Expect historical Q1 sales pattern

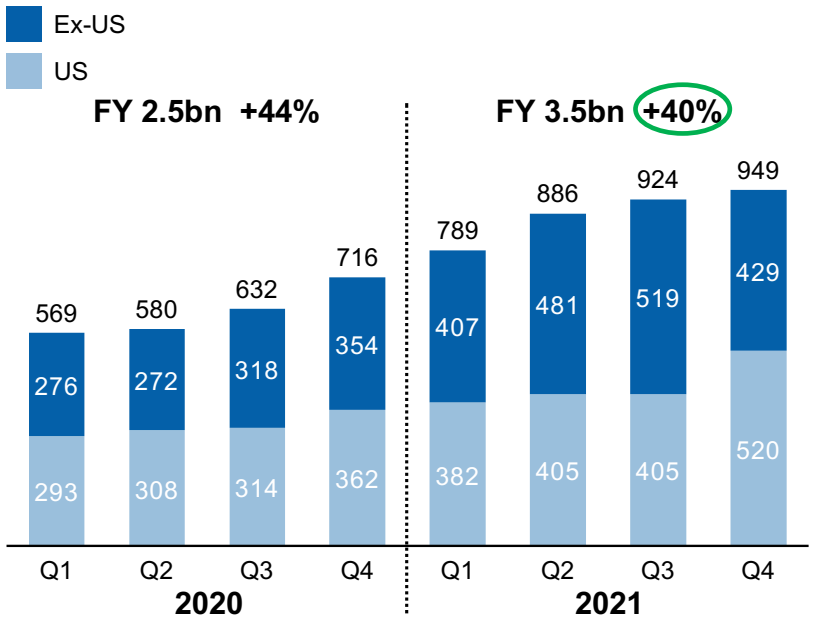
SpA – Spondyloarthritis JPsA – Juvenile psoriatic arthritis ERA – Enthesitis related arthritis PsO – Psoriasis GCA – Giant cell arteritis LN – Lupus nephritis LP – Lichen planus

Entresto® grows +40% in 2021

An essential first-choice treatment in chronic heart failure[1]

Sales evolution

USD m, % cc



Strong momentum across geographies during 2021

- US: Growth across cardiology, primary care
- Europe: Continued strong growth
- China: FY strong growth, Q4 impacted by stock compensation in anticipation of NRDL price reductions for HTN listing

Confidence in future growth across geographies

- Further patient uptake in EU and US in heart failure[1]
- Launch momentum in HTN[1] in Japan and China

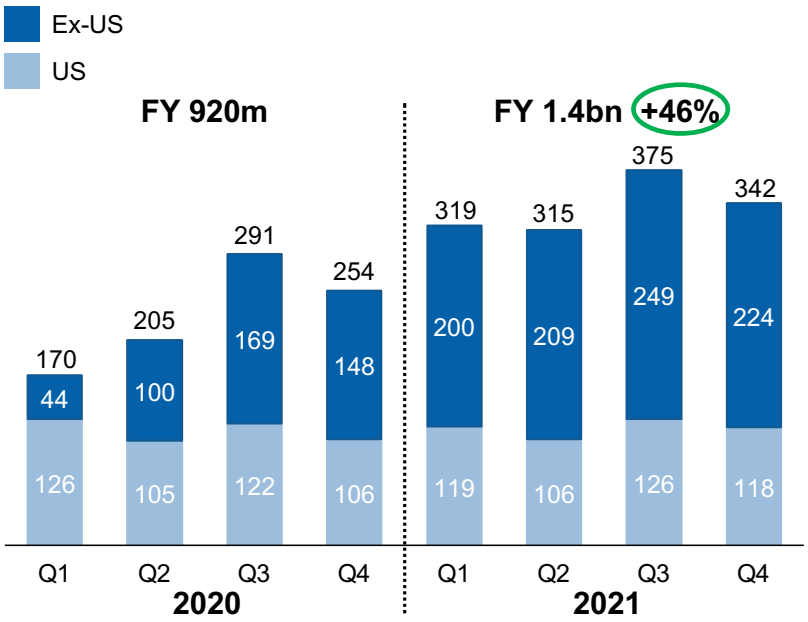
HTN – Hypertension. NRDL – National Reimbursement Drug Listing. 1. Approved indications differ by geography. Examples include "indicated to reduce the risk of cardiovascular death and hospitalization for HF in adult patients with CHF. Benefits are most clearly evident in patients with LVEF below normal." (US) HFrEF (EU) HFrEF and HTN (China and JP).

Zolgensma® grows +46% in 2021 to USD 1.4bn

Due to geographic expansion as the foundational therapy for SMA

Sales evolution

USD m, % cc



Q4 highlights

- Driven by expanding access ex-US, +58% sales
- Over 1800 patients treated worldwide[1]
- Approval now in 42 countries; access pathways in 26 countries
- Newborn screening reached ~85% in US, 20% in EU

Future outlook

- Continued growth ex-US
- Newborn screening: Goal of 38% in EU by YE22
- US: Steady US sales driven by incident patients

Advancing robust data in SMA with IT[2]

- STEER: Ph3 currently initiating treatment-naive Type 2 patients
- STRENGTH: Start H2 2022 in patients who have discontinued treatment with nusinersen and/or risdiplam

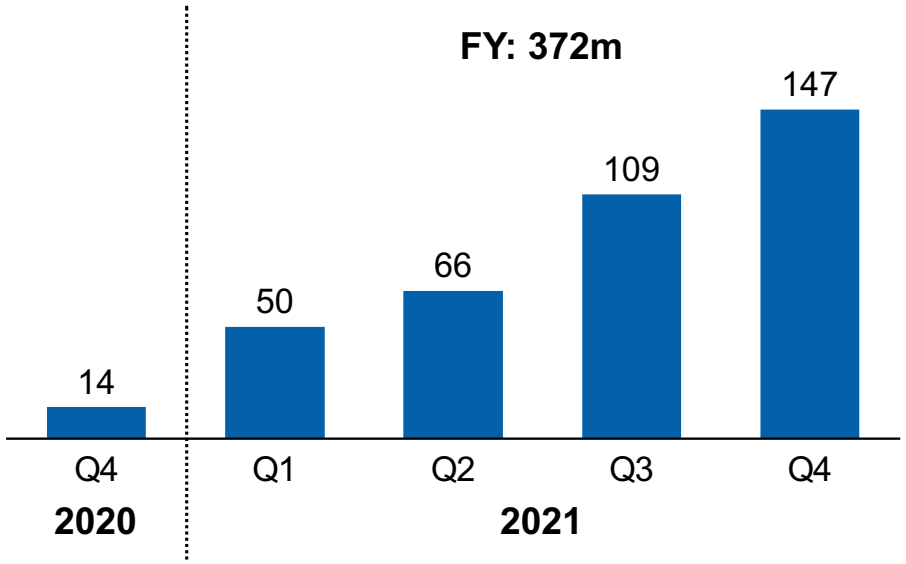
1. Commercially, via managed access programs and in clinical trials 2. With investigational OAV101 intrathecal administration

Kesimpta® launch accelerating

Clinical differentiation further enhanced by favorable vaccination data

Sales evolution

USD m, % cc



Launch progress

- ☑ Strong US sales despite COVID impacting the dynamic segment (market NBRx -12% QoQ)
- ☑ Strong access, increased demand based on compelling benefit-risk
- ☑ NBRx share **13.7%**[1], prescriber base +11%
- ☑ >8,000 people living with MS treated in US, majority naive or first switch
- ☑ Ex-US: 63 global approvals incl. China, Japan

Clinical progress

- ☑ Reassuring data in COVID vaccinated patients (ALITHIOS)[2]

1. Unadjusted share Q4. Data on file. 2. Cross AH, Delgado S, Habek M, et al. Outcomes of COVID-19 in Patients With Relapsing Multiple Sclerosis Receiving Ofatumumab: Data From the ALITHIOS Study and Post Marketing Surveillance: 37th Congress of ECTRIMS, October 13-15, 2021. Data on file from ALITHIOS, data cut off Sept 25th, 2021.

Leqvio®: US launch underway

FDA approved

- Effective and sustained LDL-C reduction[1] with twice a year maintenance dose administered by HCP
- Broad label covering 16m US ASCVD patients not at LDL-C goal
- Go-to-market model designed to overcome clinical barriers and address access, adherence and affordability
- Sales, reimbursement and medical field teams trained and deployed
- Robust network of AICs to provide acquisition and administration flexibility
- Value-based price per dose of USD 3,250
- Comprehensive patient and HCP support programs available to ensure timely access
- Product available from specialty distributors since early January
- Filed for permanent J-Code, miscellaneous J-Code for temporary use available

Expect modest initial ramp as we lay the foundation for multi-blockbuster potential

LDL-C – Low Density Lipoprotein Cholesterol ASCVD – Atherosclerotic Cardiovascular Disease AIC – Alternative Injection Center HCP – Healthcare Professional 1. Across the 6-month dosing interval.

Summary for Pharmaceuticals

- ☑ Strong performance of growth drivers (+30% FY growth), continuing portfolio rejuvenation
- ☑ Kesimpta® accelerating; Leqvio® launch underway
- ☑ In 2022, expect continued strong momentum from key growth drivers 

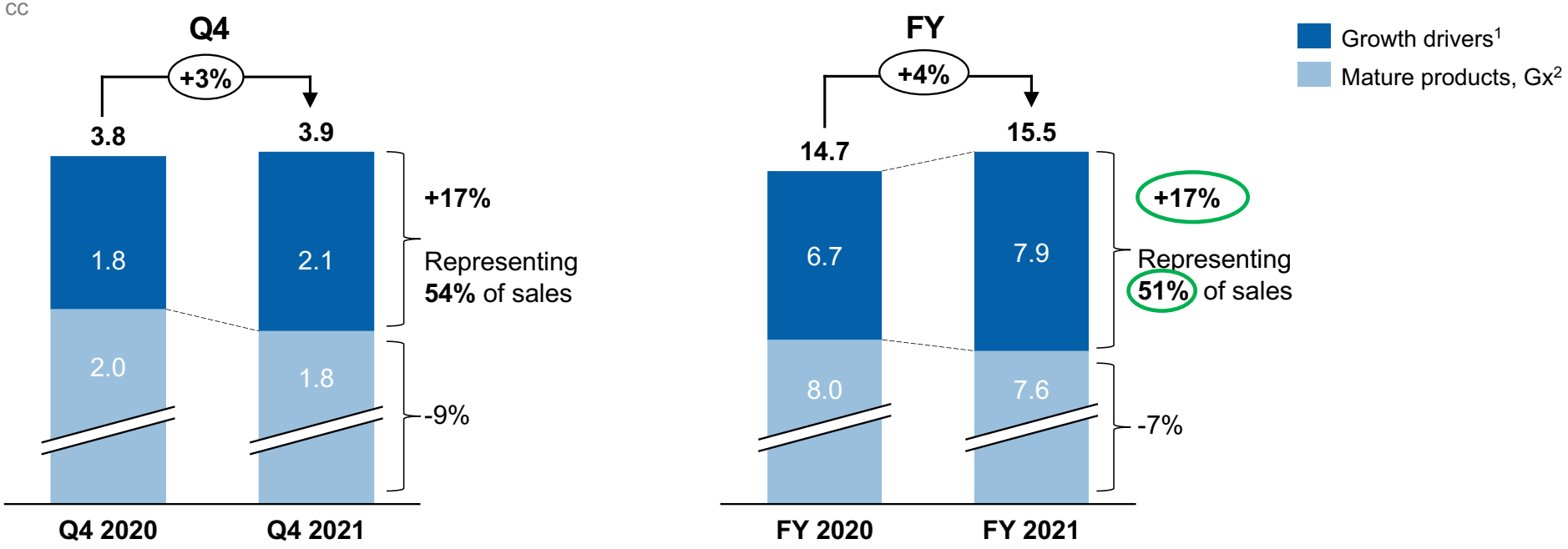
Susanne Schaffert

President, Novartis Oncology

FY Oncology sales grew +4%, overcoming Gx headwinds; portfolio rejuvenation with growth drivers representing 51% of sales

Oncology net sales

USD bn, % cc

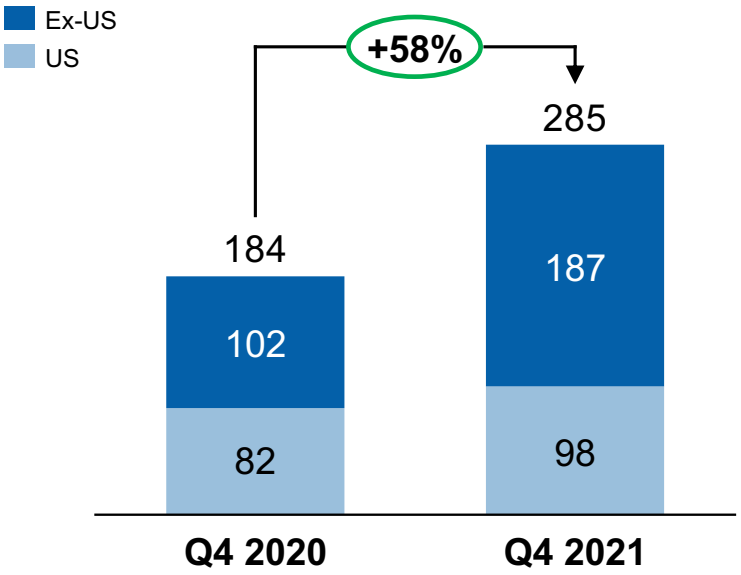


1. Include Piqray®, Adakveo®, Tabrecta® and Scemblix®, Promacta®/Revolade®, Tafinlar®+ Mekinist®, Kisqali®, Lutathera®, Kymriah® and Jakavi® (marketed by Novartis ex-US). 2. Base business – other brands. Gx include Afinitor®, Exjade® / Jadenu®, Glivec® and Sandostatin®. All % growth refers relate to cc unless otherwise stated.

Kisqali® accelerated growth in Q4 (+58%) behind MONALEESA-2 OS

Sales evolution

USD bn, % cc



Robust body of evidence supports positioning Kisqali® as standard of care (SOC) in 1L postmenopausal BC

- Positive OS results in 3 Ph3 trials, including ML-2
- The only CDK 4/6 inhibitor to demonstrate OS benefit in 1L according to NCCN guidelines

Continued growth acceleration and geographic expansion

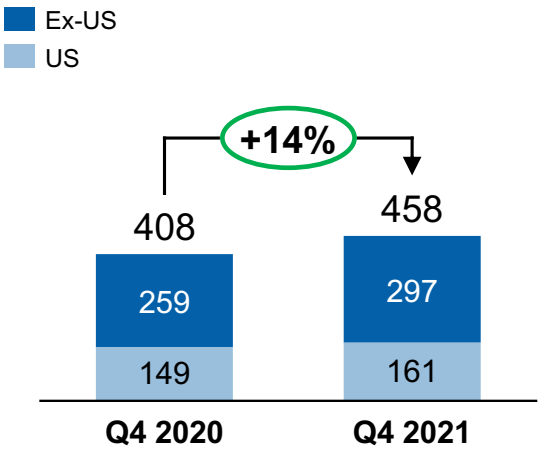
- US share gains driven by positive impact of ML-2 OS data
- 88% YoY growth ex-US reflecting strong market share gains and impact of new data
- Geographic expansion with public reimbursement recommendation in Brazil and regulatory submission in China

aBC – advanced breast cancer / eBC – early breast cancer ML – MONALEESA. In phase 3 randomized controlled trials, ribociclib + endocrine therapy has shown overall survival benefit in the first-line setting.

Tafinlar®+Mekinist®, Promacta®/Revolade® and Jakavi® with continued double-digit growth

Tafinlar®+Mekinist®

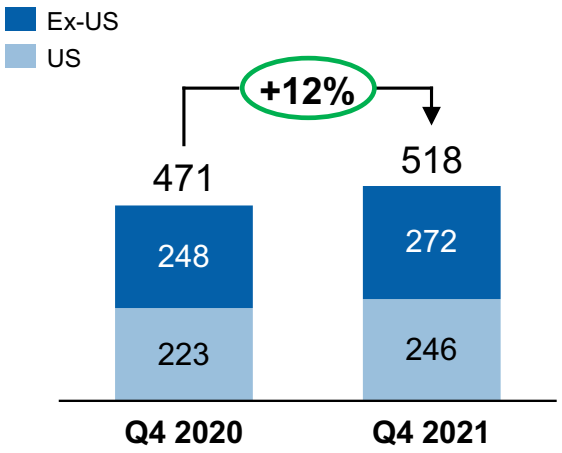
USD m, % cc



Continued growth and leadership in both adjuvant and metastatic **BRAF+ melanoma and lung**

Promacta®/Revolade®

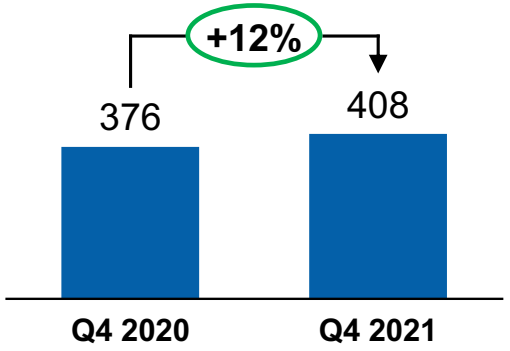
USD m, % cc



Double-digit growth in all regions driven by **sustained efficacy**, **oral** convenience and non-immunosuppressive profile

Jakavi®

USD m, % cc



Strong growth driven by earlier usage in **myelofibrosis and polycythemia vera**; further uptake expected from **GVHD** launches

GVHD – Graft-versus-host disease

Launching SCEMBLIX®, a novel STAMP inhibitor with potential to transform the standard of care in CML

SCEMBLIX® (asciminib) 20 mg, 40 mg tablets

Strong clinical profile addressing sizable medical need

- ☑ Superior clinical profile to bosutinib in late line CML[1] with early 2x improvement in MMR and >3x fewer discontinuations due to AEs
- ☑ Clinically meaningful efficacy in patients with T315I CML-CP
- ☑ ~25% of all CML patients addressable with current label
- ☑ Potential to provide another treatment option in 1L CML; Ph3 pivotal trial ongoing (filing in 2025)

US launch excellence building on CML experience

- ☑ Executing with excellence, leveraging decades of CML experience
- ☑ Patient assistance program in place with more than 50 patients registered; over 150 enrollments in managed access program
- ☑ Already included in NCCN guidelines; strong medical engagement with >50 US centers in clinical trials
- ☑ HCPs able to secure access for patients, while formulary listings are ongoing

STAMP – Specifically Targeting the ABL Myristoyl Pocket. 1. Rea D et al, Blood 2021 Nov 25;138(21).

Preparing for ^{177}Lu-PSMA-617 launch in the US, expected H1 2022

Potential new treatment paradigm in mCRPC,
based on strong Ph3 VISION data

- Significant unmet need in mCRPC[1]
- **^{177}Lu-PSMA-617 + SOC reduced risk of death by 38%**, rPFS or death by 60%, compared to SOC alone[2]
- **Median OS 15.3 months** (rPFS 8.7 months), vs. 11.3 months (rPFS 3.4 months) for SOC alone[2]
- 29.8% overall response compared to 1.7% with SOC alone[2]
- Safety profile in line with prior experience[2]
- Administration advantages (**6 one-time infusions**) over chronic therapies[2]

Laying the foundation for a steady launch,
FDA action expected H1 2022, EMA H2 2022

- ☑ Hospital capacity sufficient for launch in VISION population
- ☑ Targeting >225 treatment sites in US, ~200 sites in EU (all Lutathera® treatment sites)
- ☑ PET imaging available; ^{68}Ga-PSMA-11 imaging agent included in NCCN guidelines
- ☑ Leveraging Lutathera® team and experience; incremental FF fully recruited in US, on track in EU
- ☑ Extensive disease state education underway

Two ongoing Ph3 studies in mCRPC pre-taxane (PSMAfore) & mHSPC (PSMAddition), potential to expand eligible patient population for ^{177}Lu-PSMA-617 by 3-4x

1. VISION population: PSMA+ post ARPI and Taxane; approx. 10 months median OS on available treatments in late line mCRPC; 30% five-year survival prognosis; 80%+ of patients PSMA positive. 2. Sartor, et al. NEJM 2021; doi: 10.1056/NEJMoa2107322.

Summary for Oncology

- ☑ Continued strong execution and portfolio rejuvenation in 2021, with growth drivers up 17%
- ☑ Driving share gains with Kisqali® in CDK4/6 class, ahead of NATALEE adjuvant readout
- ☑ Focusing on launch execution for Scemblix® and ^{177}Lu-PSMA-617; preparing for next wave of launches

Harry Kirsch

Chief Financial Officer

Financial review and 2022 guidance

2021 financial results in line with guidance

Group full year guidance (Q3 earnings October 2021)
In cc

		FY 2021 vs. PY
Innovative Medicines	Sales expected **to grow mid single digit**	+6% ✓
	Core OpInc expected to **grow high single digit**	+10% ✓+
Sandoz[1]	Sales expected to **decline low to mid single digit**	-2% ✓
	Core OpInc expected to **decline mid to high teens**	-14% ✓
Group	**Sales expected to grow low to mid single digit**	**+4%** ✓
	Core OpInc expected to grow mid single digit, ahead of sales	**+6%** ✓

1. FY sales growth for Sandoz includes +1% point impact from a reclassification of contract manufacturing from other revenue to sales.

Strong Q4 with mid single digit sales and double digit core OpInc growth

Group[1] USD million	**Q4 2021**	**Change vs. PY**[2]		**FY 2021**	**Change vs. PY**	
		% USD	**% cc**		**% USD**	**% cc**
Net sales	13,229	4	6	51,626	6	4
Core operating income	3,819	9	12	16,588	8	6
Operating income	2,562	-3	-1	11,689	15	13
Net income	16,306	nm	nm	24,018	198	195
Ex. Roche divestment gain[3]	*1,734*	*-17*	*-14*	*9,446*	*17*	*15*
Core EPS (USD)	1.40	4	7	6.29	9	7
EPS (USD)	7.29	nm	nm	10.71	202	200
Ex. Roche divestment gain[3]	*0.78*	*-15*	*-13*	*4.21*	*19*	*17*
Free cash flow	3,027	-9		13,282	14	

nm – Not meaningful 1. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 49 of the Condensed Financial Report. All % growth relate to cc unless otherwise stated. 2. Q4 sales growth for Group includes +1% point impact from a reclassification of contract manufacturing from other revenue to sales. 3. See slide 54 for the reconciliation of IFRS results vs. results ex. Roche divestment gain

2021 IM core margin increased to 36.2% (+130bps)

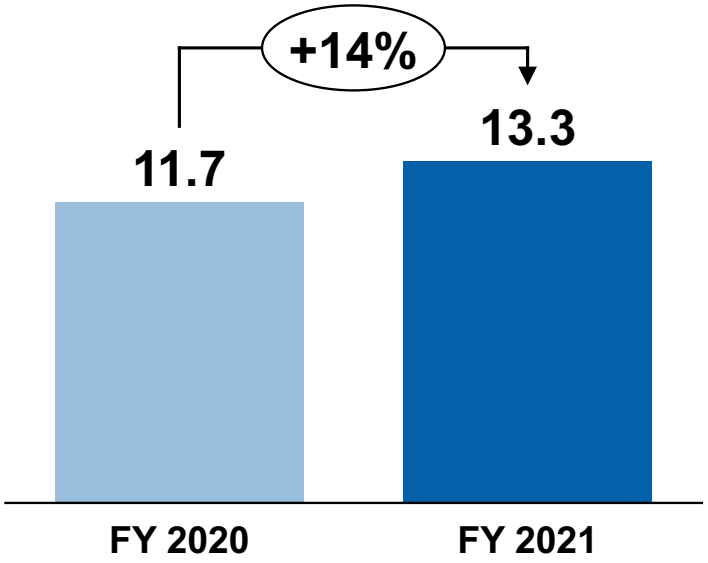
	Q4 2021				FY 2021			
	Net sales [1] change vs. PY	Core operating income[2] change vs. PY	Core margin[2]	Core margin[2] change vs. PY	Net sales[1] change vs. PY	Core operating income[2] change vs. PY	Core margin[2]	Core margin[2] change vs. PY
	(in % cc)	(in % cc)	(%)	(%pts cc)	(in % cc)	(in % cc)	(%)	(%pts cc)
Innovative Medicines	7	15	33.6	2.4	6	10	36.2	1.3
Sandoz	2	0	20.9	-0.4	-2	-14	21.4	-2.9
Group	6	12	28.9	1.6	4	6	32.1	0.5

1. Q4 sales growth for Group, IM and Sandoz includes +1% point impact from a reclassification of contract manufacturing from other revenue to sales. Sandoz FY sales growth also benefited +1% point from this reclassification. 2. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 49 of the Condensed Financial Report.

FY 2021 free cash flow grew to USD 13.3bn mainly driven by higher operating income

Group free cash flow[1]

USD bn, % USD



Key drivers vs. PY

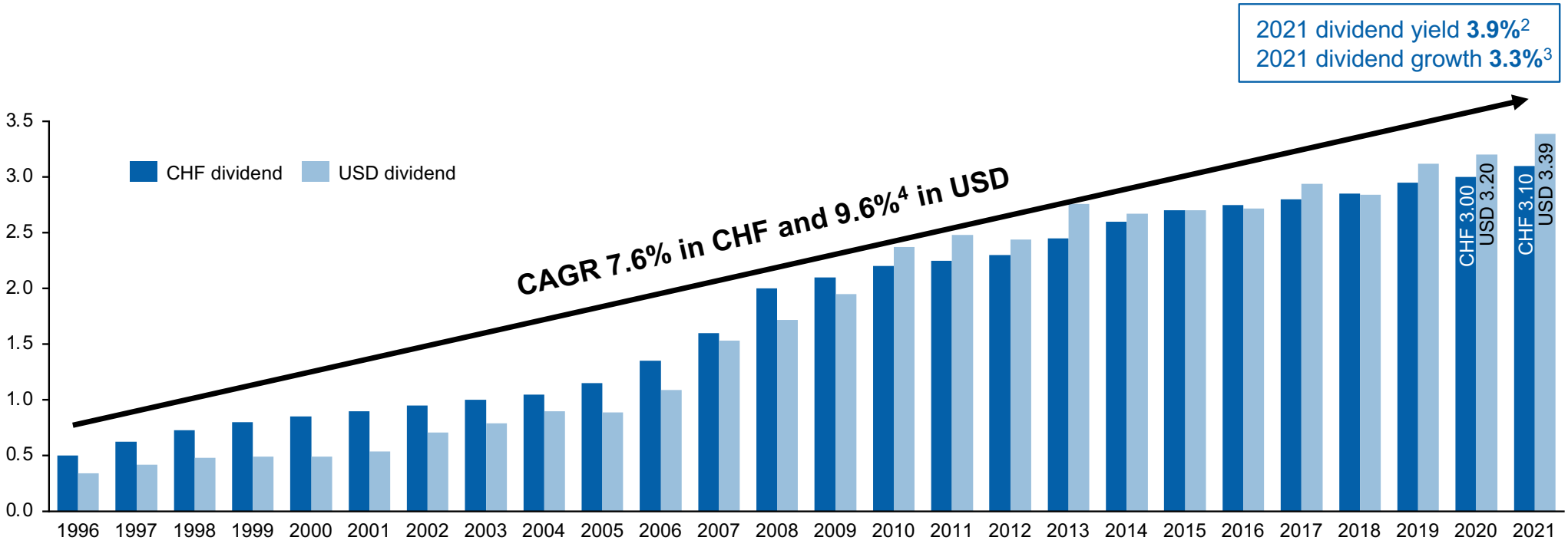
\+ Higher operating income (adjusted for non-cash items)

\+ Lower payments related to legal matters

– Tislelizumab in-licensing (upfront payment USD 650m)

1. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 49 of the Condensed Financial Report.

Novartis proposes 25th consecutive dividend increase to the AGM: 3.10 CHF / share[1]



1. Proposal to shareholders at the 2022 Annual General Meeting, taking place on March 4, 2022. 2. Based on closing share price of CHF 80.28 at end of business year 2021 (December 30, 2021). 3. In CHF. 4. Converted at historic exchange rates at the dividend payment dates as per Bloomberg; for 2021, dividend per share translated into US dollars at the December 31, 2021, rate of USD 1.093 to the Swiss franc.

2022 Novartis full year guidance

Barring unforeseen events; growth vs. PY in cc

Innovative Medicines	Sales expected to **grow mid single digit** Core OpInc expected to **grow mid to high single digit, ahead of sales**
Sandoz	Sales expected to **be broadly in line with prior year** Core OpInc expected to **decline low to mid single digit**
Group	Sales expected to **grow mid single digit** Core OpInc expected to **grow mid single digit**

Key assumptions

Our guidance assumes that we see a continuing return to normal global healthcare systems, including prescription dynamics, and that no Sandostatin® LAR generics enter in the US

FY 2022 guidance on other financial KPIs

Barring unforeseen events; growth vs. PY in cc

Group | full year guidance

vs. PY (cc)

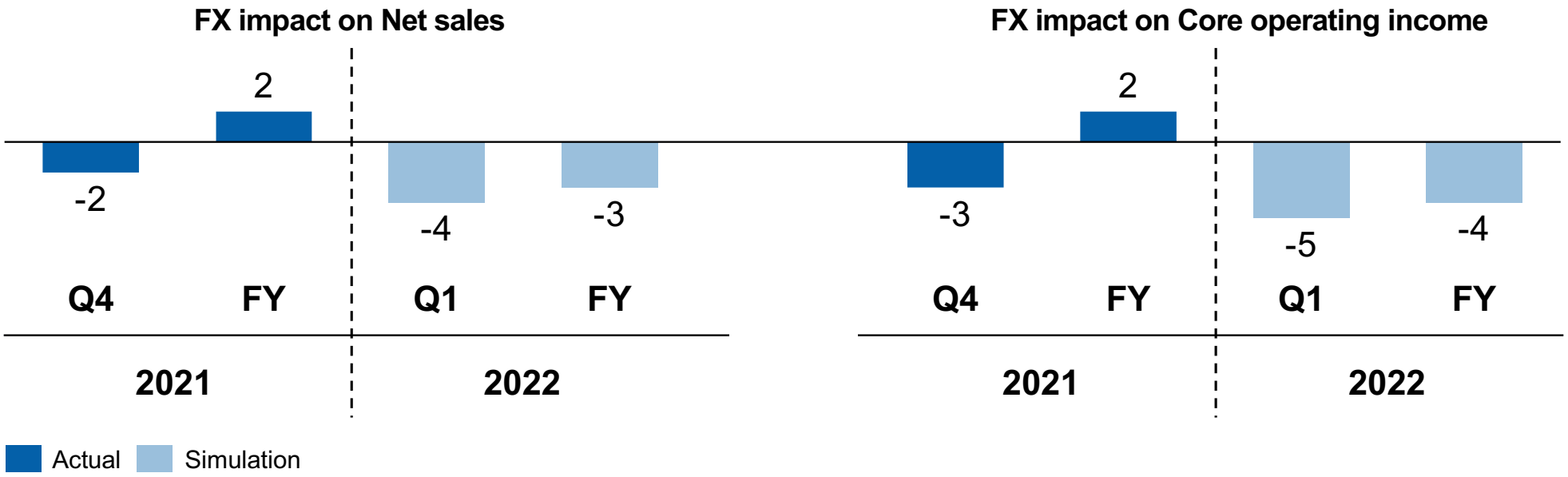
Core Net Financial Result	Expenses expected to be broadly in line vs. 2021
Core Tax Rate	Core tax rate expected to be in the 17-17.5% range: ▪ +1% vs. PY mathematical impact of Roche divestment[1] ▪ +0-0.5% due to profit mix

1. Roche net income from associated companies was recorded after tax thus lowering the average core tax rate in 2021 and prior years.

Expected currency impact for full year 2022

Currency impact vs. PY

%pts, assuming late-January exchange rates prevail in 2022



Vas Narasimhan

Chief Executive Officer

Conclusions

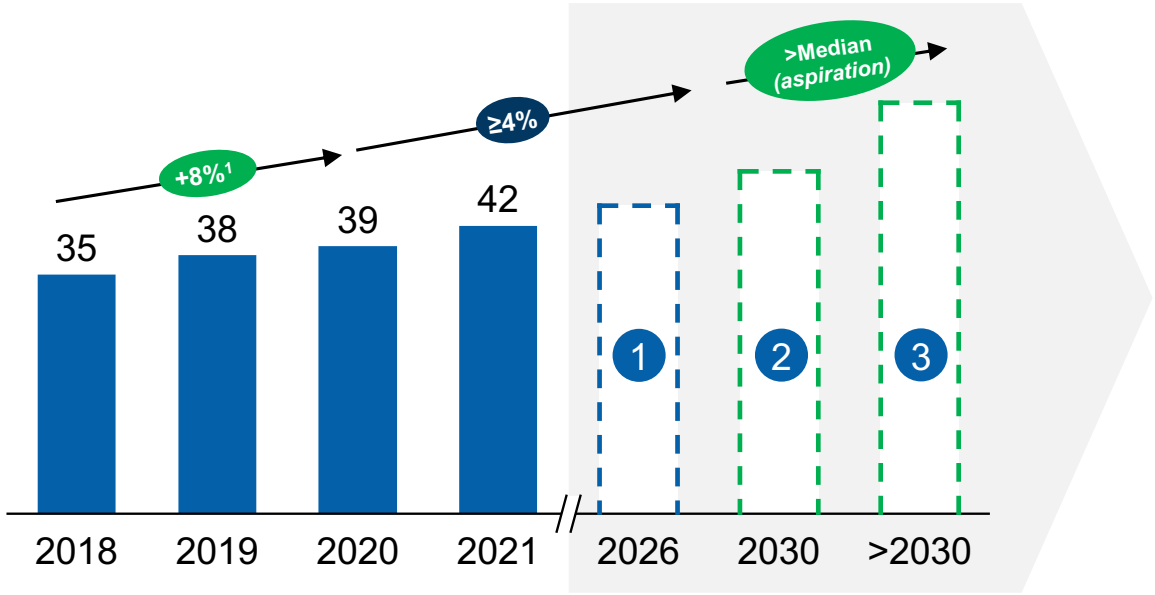
- ☑ In 2021, Novartis delivered mid single digit top-line growth, margin expansion, strong FCF
- ☑ In-market growth drivers continue to perform well across geographies, supporting our confidence in our outlook of **4%+ sales CAGR to 2026**
- ☑ Delivered important innovation milestones, e.g. Entresto®, ^{177}Lu-PSMA-617, iptacopan, Kisqali®, Leqvio®
  Focused on delivering on our pipeline: **20+ potential assets** with significant sales for approval by **2026**
- ☑ Balanced capital allocation, continuing to invest in innovation alongside returning capital to our shareholder

Appendix

Novartis is committed to driving consistent growth through 2030 and beyond

IM sales evolution

Illustrative, USD billion, % CAGR cc



1. **2020-2026 | ≥4%**
   Focused resources on key growth brands and launches, upscaling next generation engagement models

2. **2026-2030 | >peer median**
   Double-down on internal pipeline assets to unlock their full potential and add complementary BD&L

3. **>2030 | >peer median**
   Focused investments in technology platforms while staying at the forefront of innovation in small and large molecules

1. 6% in USD.

Strong FY operational performance from growth drivers

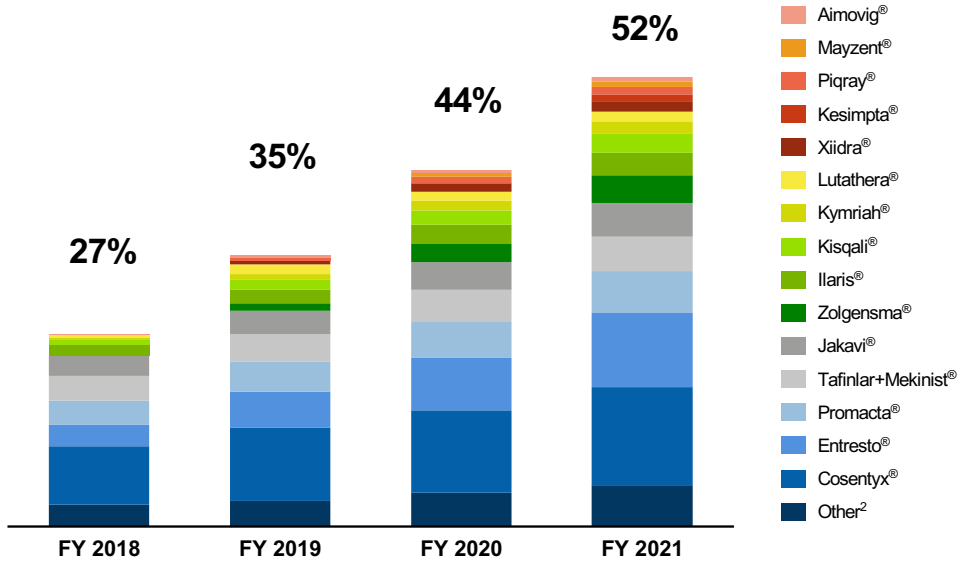
Key growth driver sales FY 2021[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto® sacubitril/valsartan	3,548	1,051	40%
Cosentyx® (secukinumab)	4,718	723	17%
zolgensma®	1,351	431	46%
Kesimpta® (ofatumumab)	372	357	nm
PROMACTA® (eltrombopag)	2,016	278	15%
JAKAVI® ruxolitinib	1,595	256	16%
KISQALI® ribociclib	937	250	36%
ILARIS® (canakinumab)	1,059	186	22%
Xolair® (omalizumab)	1,428	177	12%
Tafinlar® + Mekinist®	1,693	151	8%
KYMRIAH® (tisagenlecleucel)	587	113	22%
MAYZENT® (siponimod) tablets	281	111	65%

nm – not meaningful

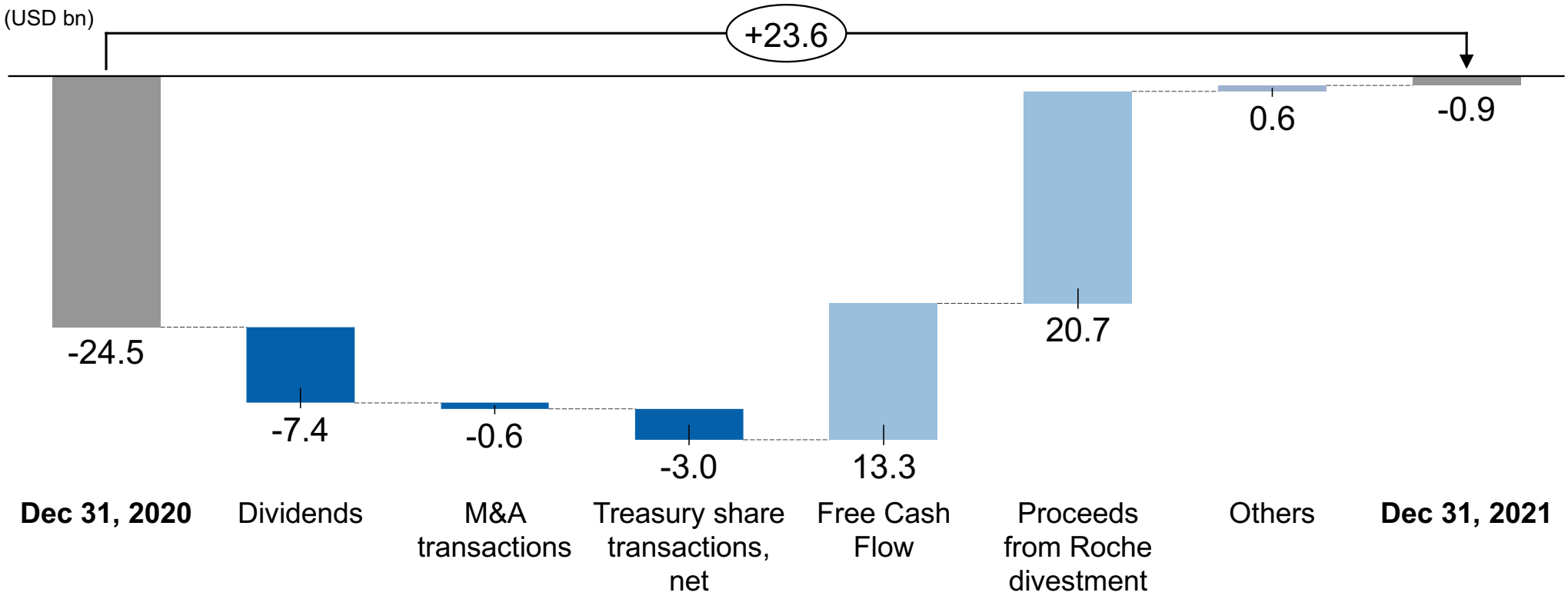
Driving portfolio rejuvenation

Key growth drivers 52% of IM sales, growing 25% vs. PY



1. Innovative Medicines division. 2. Includes Xolair®, Beovu®, Adakveo®, Tabrecta®, Luxturna®, Enerzair®, Atectura®, Leqvio® and Scemblix®. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 49 of the Condensed Financial Report.

Net debt significantly decreased by USD 23.6bn driven by proceeds from Roche divestment and strong FCF



Reconciliation of IFRS results vs. results excluding gain recognized on divestment of Roche investment

Reconciliation from IFRS reported net income and basic earnings per share, to net income and basic earnings per share excluding the gain recognized on the divestment of our investment in Roche

2021 USD million unless indicated otherwise	**IFRS results**	**Gain on divestment of our investment in Roche**	**Results excl. gain on divestment of our investment in Roche**
Operating income from continuing operations	11,689		11,689
Net income	24,018	-14,572	9,446
Total basic earnings per share (USD)	10.71	-6.50	4.21

Summary of impact of the gain recognized on the divestment of our investment in Roche in USD and constant currencies on net income and basic earnings per share

	In USD %			**In constant currencies %**		
2021	**%**	**Excluding the gain on divestment of our investment in Roche %**	**Percentage point impact**	**%**	**Excluding the gain on divestment of our investment in Roche %**	**Percentage point impact**
Net income	198	17	181	195	15	180
Total basic earnings per share (USD)	202	19	183	200	17	183

20+ potential billion USD+ pipeline assets with approval by 2026

Most are supported by high strength of evidence

Selected assets

	Strength of evidence **Moderate**	Strength of evidence **High**		
Unprobabilized peak sales **USD bn / multi-bn**	**Sabatolimab** MDS; AML	**Iptacopan** PNH; C3G; IgAN; aHUS	**Kisqali** Adj. BC (+endocrine th.) ◆	**Leqvio** Hypercholesterolemia ✓
	NIS793 PDAC; Colorectal Cancer	**Remibrutinib** CSU; MS	**YTB323**[1] 2L DLBCL	**Cosentyx** Multiple indications ◆
	Pelacarsen CVRR	**Zolgensma** SMA IT ◆	**Ianalumab** Sjogren's; SLE; AIH; Lupus Nephritis	**^{177}Lu-PSMA-617** mCRPC post & pre-taxane; mHSPC
	Canakinumab Adj. NSCLC	**Ligelizumab** FA; CINDU	**Ensovibep** Coronavirus infection	**Scemblix** 3L+ CML; 1L CML
	Ociperlimab[1] NSCLC			**Tislelizumab** Multiple indications
	UNR844 Presbyopia			**Piqray (alpelisib)** PROS; HER2+ adv BC; TNBC; ovarian cancer ◆
	Libvatrep (SAF312) Chronic Ocular Surface Pain			
	TNO155, JDQ443[2] NSCLC; Colorectal Cancer; Combos			
Unprobabilized peak sales **up to USD 1bn**		**Lutathera** 1L G2/G3 NET ◆	**Kymriah** r/r Follicular Lymphoma ◆	**Beovu** DME ◆
			Tafinlar/Mekinist Solid Tumor Agnostic ◆	**Jakavi** SR GvHD ◆

Most advanced and key indication(s) **approved by 2026**

- Submission
- Phase III
- Phase II
- ◆ LCM
- ✓ Approved

1. BeiGene option deal. 2. Ph3 to start in 2022. Assets are shown in the phase of the most advanced indication (listed first). Value based on the total of the listed indication(s). Strength of evidence based on the most advanced indication: High if in Ph3 or when Ph2 results available for the same MoA in the lead indication.

Key milestones of pipeline assets with significant sales potential with approval by 2026

Selected assets, most advanced and key indication(s) approved by 2026

High strength of evidence	2022	2023	2024	2025	2026
Iptacopan PNH	Ph3 readout	Submission			
Iptacopan C3G		Ph3 read/sub			
Iptacopan aHUS			Ph3 readout	Submission	
Iptacopan IgAn		Ph3 read/sub			
Remibrutinib CSU			Ph3 read/sub		
Remibrutinib MS				Ph3 read/sub	
Zolgensma SMA IT			Ph3 readout	Submission	
Ligelizumab CSU	Ph3 data in evaluation				
Ligelizumab CINDU			Ph3 readout	Submission	
Ligelizumab Food Allergy				Ph3 read/sub	
Kisqali	Ph3 readout[1]	Submission			
YTB323 2L DLBCL	Ph3 start		Ph3 read/sub		
Ianalumab Sjögren's	Ph3 start				Ph3 read/sub
Ianalumab LN	Ph3 start				Ph3 read/sub
Cosentyx HS	Submission				
Cosentyx Lichen Planus	Ph2 readout			Submission	
Cosentyx AS H2H	Ph3 readout	Submission			
Cosentyx GCA			Ph3 read/sub		
^{177}Lu-PSMA-617 mCRPCR post tax	Approval				
^{177}Lu-PSMA-617 mCRPCR pre tax	Ph3 readout[1]	Submission			
^{177}Lu-PSMA-617 mHSPC			Ph3 read/sub		

NME Lead

High strength of evidence	2022	2023	2024	2025	2026
Ensovibep COVID	Submission				
Scemblix	Approval				
Scemblix CML 1L			Ph3 readout	Submission	
Alpelisib PROS	Approval				
Piqray Ovarian Cancer		Ph3 read/sub			
Piqray TNBC		Ph3 read/sub			
Piqray HER2+ adv BC				Ph3 read/sub	
Tislelizumab	Submissions and approvals of several indications				

Moderate strength of evidence	2022	2023	2024	2025	2026
Sabatolimab MDS	Ph2 readout	Ph3 readout	*Submission in 2022/23*		
Sabatolimab AML		Ph3 readout	Submission		
NIS793 PDAC				Ph3 read/sub	
Pelacarsen CVRR				Ph3 read/sub	
Canakinumab Adj. NSCLC	Ph3 readout	Submission			
Ociperlimab	Ph3 start	*BeiGene option deal*			
UNR844 Presbyopia	Ph2 readout		Submission		
Libvatrep COSP		Ph2 readout			Submission
JDQ443 NSCLC	Ph3 start		Submission		
JDQ443+TNO155 NSCLC		Ph3 start			Submission

1. Event driven, could move to early 2023.

2021 key pipeline milestones[1]

✓ **Achieved**
✓ **Mixed results**
✓ **Readout not supportive**

	H1 2021			H2 2021		
Regulatory decisions and opinions	Entresto®	HFpEF (US)	✓	Cosentyx®	Pediatric psoriasis (US / CN / JP)	✓
	Kesimpta®	Relapsing MS (EU / JP)	✓			
Major expected submissions	Leqvio®	Hyperlipidemia (US)[2]	✓	Asciminib (ABL001)	CML 3L (JP)	✓
	Jakavi®	Acute and chronic GvHD (EU / JP)	✓	Beovu®	DME (JP)	✓
	Tabrecta®	NSCLC (EU)	✓	Alpelisib (BYL719)	PROS (US)	✓
	Beovu®	DME (US / EU)	✓ H2	Kymriah®	r/r Follicular lymphoma (US / EU / JP)	✓
	Asciminib (ABL001)	CML 3L (US /EU)	✓	^{177}Lu-PSMA-617	mCRPC (US / EU)	✓
	Cosentyx®	JIA (US /EU)	✓	Tislelizumab (VDT482)	2L esophageal cancer (US)	✓
				Tislelizumab (VDT482)	NSCLC (EU / US)	H1 2022[3]
Major expected trial readouts*	Iptacopan (LNP023)	Ph2 - IgAN	✓	Canakinumab (ACZ885)	Ph3 - NSCLC 1L	✓[9]
	Iptacopan (LNP023)	Ph2 - C3G	✓ H2	ECF843	Ph2 - Dry eye	✓[4]
	Entresto®	Ph3 - Post-AMI	✓[5]	Ligelizumab (QGE031)	Ph3 - CSU	✓[6]
	Canakinumab (ACZ885)	Ph3 - NSCLC 2L	✓[7]	Kisqali®	Ph3 - aBC (MONALEESA-2 OS)	✓
	^{177}Lu-PSMA-617	Ph3 - mCRPC	✓	Remibrutinib (LOU064)	Ph2 - CSU	✓
	Cosentyx®	Ph3 - JIA	✓	Cosentyx®	Ph3 - HS	✓
				Sabatolimab (MBG453)	Ph2 - MDS[8]	
				Kymriah®	Ph3 - aNHL 2L	✓[7]

*Achieved = on-time readout of data, irrespective of trial outcome. 1. 2021 Key milestone table may evolve based on read-out outcomes as well as BD&L activities. 2. Resubmitted to FDA. 3. H1 2022 EU submission, H2 2022 2L US submission. 4. Program discontinued in broad population of moderate to severe DED. 5. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed. No submission planned. 6. Ligelizumab demonstrated superiority compared with placebo PEARL 1 and PEARL 2 trials, but not versus omalizumab, further evaluating PEARL data. 7. Negative readout. 8. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial. 9. Ph3 study did not meet primary endpoints. PFS and OS trends support further evaluation with additional analyses ongoing.

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
Oncology	49	27	6	82
Pharmaceuticals	58	25	2	85
Cardiovascular, Renal, Metabolism	5	6	0	11
Immunology, Hepatology, Dermatology	26	9	1	36
Neuroscience	6	5	0	11
Ophthalmology	5	1	1	7
Respiratory & Allergy	8	3	0	11
Global Health	8	1	0	9
Biosimilars	0	2	0	2
Total	107	54	8	169

Novartis pipeline in Phase 1 (1 of 2)

32 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors
AAA817	Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer
ADPT01	ADPT01	-	Colorectal cancer (combos)
ADPT03	ADPT03	BCL11A	Sickle cell anemia
DFF332	DFF332	HIF2A inhibitor	Renal cell carcinoma
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers
HDM201	HDM201 + MBG453, venetoclax	MDM2 inhibitor	Haematological malignancy
IAG933	IAG933	-	Mesothelioma
JBH492	JBH492	-	Haematological malignancy
JDQ443	JDQ443	KRAS Inhibitor	KRAS G12C mutated solid tumors
JEZ567	JEZ567	CD123 CAR-T	Acute myeloid leukaemia
KAZ954	KAZ954	-	Solid tumors
LXF821	LXF821	EGFR CAR-T	Glioblastoma multiforme
LXH254	LXH254	cRAF inhibitor	NSCLC (combos)
MAK683	MAK683	EED inhibitor	Cancers
MBG453	sabatolimab	TIM3 antagonist	Low risk myelodysplastic syndrome
MCM998	MCM998, LXG250	BCMA CAR-T, CD19 CAR-T	Multiple myeloma
MIK665	MIK665	MCL1 inhibitor	Acute myeloid leukaemia (combo)
NIS793	NIS793, spartalizumab	TGFB1 inhibitor	Solid tumors
NIZ985	NIZ985, spartalizumab	IL-15 agonist	Solid tumors
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors
PDR001	spartalizumab	PD1 inhibitor	Solid tumors (combo)
PHE885	PHE885	BCMA cell therapy	Multiple Myeloma
TNO155	TNO155	SHP2 inhibitor	Solid tumors (combo)
			Solid tumors (combo)
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Haematological malignancy
VOB560	VOB560	-	Cancers
VPM087	gevokizumab	IL-1 beta antagonist	Colorectal cancer, 1st line
WNT974	WNT974 + spartalizumab	Porcupine inhibitor	Solid tumors
WVT078	WVT078	-	Multiple myeloma
YTB323	YTB323	CD19 CAR-T	DLBCL and adult ALL

Novartis pipeline in Phase 1 (2 of 2)

32 lead indications

Lead indication

Immunology

Code	Name	Mechanism	Indication(s)
FIA586	FIA586	-	Non-alcoholic steatohepatitis (NASH)
MHS552	MHS552	-	Autoimmune indications
MHV370	MHV370	-	Systemic lupus erythematosus
NGI226	NGI226	-	Tendinopathy

Neuroscience

Code	Name	Mechanism	Indication(s)
NIO752	NIO752	Tau antagonist	Progressive supranuclear palsy

Ophthalmology

Code	Name	Mechanism	Indication(s)
MHU650	MHU650	-	Diabetic eye diseases

Respiratory & Allergy

Code	Name	Mechanism	Indication(s)
LTP001	LTP001	-	Respiratory diseases
NCJ424	NCJ424	-	Respiratory diseases

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
MBL949	MBL949	-	Obesity related diseases

Global Health

Code	Name	Mechanism	Indication(s)
EYU688	EYU688	NS4B inhibitor	Dengue
KAF156	ganaplacide	-	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated

Novartis pipeline in Phase 2

29 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)		
AAA601	Lutathera®	Radioligand therapy target SSTR	GEPNET, pediatrics		
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 2L, pediatrics		
BLZ945	BLZ945	CSF-1R inhibitor	Solid tumors		
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	HGG/LGG, pediatrics		
INC280	Tabrecta®	Met inhibitor	Non-small cell lung cancer (Combo)		
INC424	Jakavi®	JAK1/2 inhibitor	Myelofibrosis (combo)	Acute GVHD, pediatrics	Chronic GVHD, pediatrics
JDQ443	JDQ443	KRAS inhibitor	NSCLC (combo)		
LNP023	iptacopan	CFB inhibitor	Autoimmune cytopenias		
LXH254	LXH254	cRAF inhibitor	Melanoma (combo)		
MBG453	sabatolimab	TIM3 antagonist	Unfit acute myeloid leukaemia		
			Acute myeloid leukaemia, maintenance		
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers		
NIS793	NIS793	TGFB1 inhibitor	Colorectal cancer (Combos)		
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics		
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell anaemia with crisis, pediatrics		
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)		

Immunology

Code	Name	Mechanism	Indication(s)			
ADPT02	ADPT02	-	Non-alcoholic steatohepatitis (Combos)			
AIN457	Cosentyx®	IL17A inhibitor	Lichen planus			
CFZ533	iscalimab	CD40 inhibitor	Sjögren's	Liver Tx	Hidradenitis suppurativa	
CMK389	CMK389	IL-18 inhibitor	Atopic dermatitis			
DFV890	DFV890	NLRP3 inhibitor	Osteoarthritis			
			Familial cold auto-inflammatory syndrome			
LJN452	tropifexor + licogliflozin	FXR agonist	Non-alcoholic steatohepatitis (Combos)			
LNA043	LNA043	ANGPTL3 agonist	Knee osteoarthritis	Osteoarthritis (combos)		
LOU064	remibrutinib	BTK inhibitor	Sjögren's			
LRX712	LRX712	-	Osteoarthritis			
LYS006	LYS006	Anti-inflammatory	Acne	Colitis ulcerative	Hidradenitis suppurativa	
MAS825	MAS825	-	NLRC4-GOF indications	Hidradenitis suppurativa		
MHV370	MHV370	-	Sjögren's			
VAY736	ianalumab	BAFF-R inhibitor	Sjögren's	Autoimmune hepatitis		
			Systemic lupus erythematosus			

Ophthalmology

Code	Name	Mechanism	Indication(s)		
CPK850	CPK850	RLBP1 AAV	Retinitis pigmentosa		
LKA651	LKA651	EPO inhibitor	Diabetic retinopathy		
SAF312	libvatrep	TRPV1 antagonist	Chronic ocular surface pain		
UNR844	UNR844	Reduction of disulfide bonds	Presbyopia		

Respiratory & Allergy

Code	Name	Mechanism	Indication(s)		
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis		
CSJ117	CSJ117	TSLP inhibitor	Asthma	Chronic obstructive pulmonary disease	
QBW251	icenticaftor	CFTR potentiator	Chronic obstructive pulmonary disease	Bronchiectasis	
QMF149	Atectura®	Combo	Asthma, pediatrics		

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)	
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis	Type 1 diabetes mellitus
HSY244	HSY244	-	Atrial fibrillation	
LNP023	iptacopan	CFB inhibitor	Membranous nephropathy	

Neuroscience

Code	Name	Mechanism	Indication(s)
ADPT06	ADPT06	-	Cognitive impairment
BLZ945	BLZ945	CSF-1R inhibitor	Amyotrophic lateral sclerosis
DLX313[2]	DLX313	Alpha-synuclein Inhibitor	Parkinson's disease
LMI070	branaplam	mRNA splicing modulator	Huntington's disease
MIJ821	MIJ821	NR2B negative allosteric modulator	Acute depression

Global Health

Code	Name	Mechanism	Indication(s)	
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe	Malaria, uncomplicated
KAF156	ganaplacide	-	Malaria, uncomplicated	
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis	
SKO136	ensovibep	Multi-specific DARPin	Corona virus infection	

1. Clinical hold lifted. 2. =UCB0599.

Novartis pipeline in Phase 3

8 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
AAA617	^{177}Lu-PSMA-617	Radioligand therapy target PSMA	mCRPC, pre-taxane
			Metastatic hormone sensitive prostate cancer (mHSPC)
AAA601[1)]	Lutathera®	Radioligand therapy target SSTR	Gastroenteropancreatic neuroendocrine tumors, 1st line in G2/3 tumors (GEP-NET 1L G3)
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 1st line
ACZ885	canakinumab	IL-1b inhibitor	NSCLC, adjuvant
BYL719	Piqray®	PI3Kα inhibitor	HER2+ adv BC / Triple negative breast cancer / Ovarian cancer
CTL019	Kymriah®	CD19 CAR-T	1L high risk acute lymphocytic leukaemia, pediatrics & young adults
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	Thyroid cancer
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	r/r Severe aplastic anemia
INC280	Tabrecta®	Met inhibitor	Non-small cell lung cancer
JDQ443	JDQ443	KRAS inhibitor	2/3L Non-small cell lung cancer
LEE011	Kisqali®	CDK4 Inhibitor	HR+/HER2- BC (adj)
LNP023	iptacopan	CFB inhibitor	Paroxysmal nocturnal haemoglobinuria
			Atypical haemolytic uraemic syndrome
MBG453	sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
NIS793	NIS793	TGFB1 inhibitor	Pancreatic cancer
VDT482	tislelizumab	PD1 inhibitor	1L Nasopharyngeal Carcinoma / Non-small cell lung cancer
			1L ESCC / 1L Gastric cancer
			1L Hepatocellular Carcinoma / Localized ESCC
			1L Bladder Urothelial Cell Carcinoma / 1L Small Cell Lung Cancer
YTB323	YTB323	CD19 CAR-T	2L Diffuse large B-cell lymphoma[3)]

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Lupus Nephritis / AS H2H / Hidradenitis suppurativa
			Psoriatic arthritis (IV formulation)
			Axial SpA (IV formulation)
			Giant cell arteritis
QGE031	ligelizumab	IgE inhibitor	Chronic spontaneous urticaria
			Chronic inducible urticarial (CINDU)
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria

Neuroscience

Code	Name	Mechanism	Indication(s)
AMG334	Aimovig®	CGRPR antagonist	Migraine, pediatrics
BAF312	Mayzent®	S1P1,5 receptor modulator	Multiple sclerosis, pediatrics
LOU064	remibrutinib	BTK inhibitor	Multiple sclerosis
OAV101	AVXS-101	SMN1 gene replacement therapy	SMA IT administration
OMB157	Kesimpta®	CD20 Antagonist	Multiple sclerosis, pediatrics

Respiratory & Allergy

Code	Name	Mechanism	Indication(s)
IGE025	Xolair®	IgE inhibitor	Food allergy / Auto-injector
QGE031	ligelizumab	IgE inhibitor	Food allergy

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR-LDLC / Hyperlipidemia, pediatrics
LCZ696	Entresto®	Angiotensin receptor/neprilysin inhibitor	Congestive heart failure, pediatrics[2)]
LNP023	iptacopan	CFB inhibitor	IgA nephropathy
			C3 glomerulopathy
TQJ230	Pelacarsen	ASO targeting Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a) (CVRR-Lp(a))

Biosimilars

Code	Name	Mechanism	Indication(s)
GP2411	denosumab	anti RANKL mAb	Osteoporosis (same as originator)
SOK583	aflibercept	VEGF inhibitor	Ophthalmology indication (as originator)

Ophthalmology

Code	Name	Mechanism	Indication(s)
RTH258	Beovu®	VEGF inhibitor	Diabetic retinopathy

Global Health

Code	Name	Mechanism	Indication(s)
COA566	Coartem®	-	Malaria, uncomplicated (<5kg patients)

1. ^{177}Lu-dotatate in US. 2. Approved in US. 3. Ph3 to be initiated in 2022.

Novartis pipeline in registration

2 lead indication

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
AAA617	^{177}Lu-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer, post-taxane
BYL719	alpelisib	PI3Kα inhibitor	PIK3CA-related overgrowth spectrum
CTL019	Kymriah®	CD19 CAR-T	r/r Follicular lymphoma
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD
			Chronic GVHD
VDT482	tislelizumab	PD1 inhibitor	2L ESCC

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Cosentyx 300mg auto-injector and pre-filled syringe

Ophthalmology

Code	Name	Mechanism	Indication(s)
RTH258	Beovu®	VEGF inhibitor	Diabetic macular edema

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

LEAD INDICATIONS

2022

- **ligelizumab**[1] — Lead — QGE031, CSU
- **sabatolimab**[2] — Lead — MBG453, HR-MDS
- **ensovibep** — Lead — SKO136, COVID19

2023

- **iptacopan** — Lead — LNP023, PNH

2024

- **JDQ443** — Lead — JDQ443, 2/3L NSCLC (mono)
- **remibrutinib** — Lead — LOU064, CSU
- **UNR844** — Lead — Presbyopia
- **YTB323** — Lead — 2L Diffuse large B-cell lymphoma

2025

- **icenticaftor** — Lead — QBW251, COPD
- **NIS793** — Lead — 1L Pancreatic cancer
- **pelacarsen** — Lead — TQJ230, CVRR-Lp(a)

≥2026

- **^{177}Lu-NeoB** — Lead — AAA603, Multiple Solid Tumors
- **branaplam** — Lead — LMI070, Huntington's disease
- **cipargamin** — Lead — KAE609, Malaria severe
- **CPK850** — Lead — RP
- **CSJ117** — Lead — Asthma
- **gevokizumab** — Lead — VPM087, 1st line CRC / 1st line RCC
- **ganaplacide** — Lead — KAF156, Malaria uncomplicated
- **iscalimab** — Lead — CFZ533, Sjögren's syndrome
- **ianalumab** — Lead — VAY736, Sjögren's syndrome
- **libvatrep** — Lead — SAF312, COSP
- **LNA043** — Lead — Knee osteoarthritis
- **LXE408** — Lead — Visceral leishmaniasis
- **LXH254** — Lead — Solid tumors (combos)
- **MIJ821** — Lead — Acute depression
- **TNO155** — Lead — Solid tumors
- **tropifexor&licogliflozi** — Lead — LJN452, NASH (combos)

NEW INDICATIONS

2022

- **tislelizumab** — LCM — VDT482, 1L Nasopharyngeal Carcinoma
- **tislelizumab** — LCM — VDT482, NSCLC

2023

- **177Lu-PSMA-617** — LCM — AAA617, Pre-taxane
- **iptacopan** — LCM — LNP023, C3G
- **iptacopan** — LCM — LNP023, IgAN
- **tislelizumab** — LCM — VDT482, 1L Gastric Cancer
- **tislelizumab** — LCM — VDT482, 1L ESCC
- **tislelizumab** — LCM — VDT482, Localized ESCC
- **tislelizumab** — LCM — VDT482, 1L Hepatocellular Carcinoma

2024

- **177Lu-PSMA-617** — LCM — AAA617, mHSPC
- **sabatolimab** — LCM — MBG453, Unfit AML
- **tislelizumab** — LCM — VDT482, 1L Small Cell Lung Cancer
- **tislelizumab** — LCM — VDT482, 1L Bladder Urothelial Cell Carcinoma

2025

- **asciminib** — LCM — ABL001, CML 1L
- **iptacopan** — LCM — LNP023, aHUS
- **ligelizumab** — LCM — QGE031, Food allergy
- **ligelizumab** — LCM — QGE031, CINDU
- **remibrutinib** — LCM — LOU064, Multiple sclerosis

≥2026

- **asciminib** — LCM — ABL001, CML, 2L, pediatrics
- **cipargamin** — LCM — KAE609, Malaria uncomplicated
- **JDQ443** — LCM — JDQ443, NSCLC (combo)
- **ianalumab** — LCM — VAY736, AIH
- **iptacopan** — LCM — LNP023, iMN
- **iscalimab** — LCM — CFZ533, Liver Tx
- **remibrutinib** — LCM — LOU064, Sjögren's syndrome

1. Ph3 data in evaluation. 2. Filing opportunity in 2022 / 2023, based on PFS and/or OS outcomes from a dual approach based on parallel Phase 2 and Phase 3 trials.

Novartis submission schedule

Supplementary indications for existing brands

2022

Cosentyx LCM
secukinumab, AIN457
PsA IV

Cosentyx LCM
secukinumab, AIN457
AS H2H

Cosentyx LCM
secukinumab, AIN457
Hidradenitis suppurativa

Entresto EU[1] LCM
sacubitril/valsartan, LCZ696
Pediatric CHF

Tafinlar + Mekinist LCM
dabrafenib + trametinib, DRB436
HGG/LGG - Pediatrics

Xolair LCM
omalizumab, IGE025
Auto-injector

2023

canakinumab LCM
ACZ885
Adjuvant NSCLC

Cosentyx LCM
secukinumab, AIN457
AS IV

denosumab BioS
GP2411
anti RANKL mAb

Kisqali LCM
ribociclib, LEE011
HR+/HER2- BC (adj)

Lutathera LCM
^{177}Lu-oxodotreotide[2]
GEP-NET 1L G3

Piqray LCM
alpelisib, BYL719
TNBC

Piqray LCM
alpelisib, BYL719
Ovarian cancer

Promacta LCM
eltrombopag, ETB115
r/r severe aplastic anemia

Xolair LCM
omalizumab, IGE025
Food allergy

2024

Adakveo LCM
SEG101
Sickle cell anaemia with crisis ped

Coartem LCM
artemether + lumefantrine, COA566
Malaria uncompl., formula for <5kg

Cosentyx LCM
secukinumab, AIN457
GCA

Jakavi LCM
ruxolitinib, INC424
Pediatrics Acute GVHD

Jakavi LCM
ruxolitinib, INC424
Pediatrics Chronic GVHD

Leqvio LCM
KJX839
Ped Hyoerlipidemia

Tafinlar + Mekinist LCM
dabrafenib + trametinib, DRB436
Thyroid cancer

2025

aflibercept BioS
SOK583
Neovascular age-related macular degeneration

Beovu LCM
brolucizumab, RTH258
Diabetic retinopathy

Cosentyx LCM
secukinumab, AIN457
Lichen Planus

Piqray LCM
alpelisib, BYL719
HER2+ adv BC

Zolgensma LCM
AVXS-101 OAV101
SMA IT

≥2026

Atectura LCM
indacaterol + mometasone, QMF149
Asthma, pediatrics

Jakavi LCM
ruxolitinib, INC424
Myelofibrosis (combination)

Leqvio LCM
KJX839
CVRR-LDLC

Aimovig LCM
erenumab, AMG334
Pediatric Migraine

Kesimpta[3] LCM
ofatumumab
Multiple sclerosis, pediatrics

Mayzent[4] LCM
siponimod, BAF312
Multiple sclerosis, pediatrics

Cosentyx LCM
secukinumab, AIN457
Lupus Nephritis

Kymriah LCM
tisagenlecleucel, CTL019
1L high risk ALL, pediatrics & young adults

Rydapt LCM
midostaurin, PKC412
Acute myeloid leukemia, pediatrics

1. Approved in US. 2. 177Lu-dotatate in US. 3. Kesimpta and Mayzent: pediatric study in multiple sclerosis run in conjunction (NEOS).

Clinical Trials Update

Includes selected ongoing or recently concluded global trials of Novartis development programs/products which are in confirmatory development or marketed (typically Phase 2b or later).

For further information on all Novartis clinical trials, please visit: www.novartis.com/clinicaltrials.

Cardiovascular, Renal and Metabolic

Entresto® - Angiotensin receptor/neprilysin inhibitor

Study	NCT02678312 PANORAMA HF (CLCZ696B2319)	NCT02884206 PERSPECTIVE (CLCZ696B2320)
Indication	Heart failure in pediatric patients	Heart failure
Phase	Phase 3	Phase 3
Patients	360	592
Primary Outcome Measures	Part 1: Pharmacodynamics and pharmacokinetics of sacubitril/valsartan LCZ696 analytes Part 2: Efficacy and safety compared with enalapril	Change from baseline in the CogState Global Cognitive Composite Score (GCCS)
Arms Intervention	Part 1: Sacubitril/valsartan 0.8 mg/kg or 3.1 mg/kg or both; 0.4 mg/kg or 1.6 mg/kg or both (single doses). Part 2: enalapril/placebo 0.2 mg/kg bid (ped. formulation 1mg/ml) and adult formulation (2.5, 5, 10 mg bid); Sacubitril/valsartan (LCZ696)/placebo: Ped. formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid)	Sacubitril/valsartan 50, 100, and 200 mg bid with placebo of valsartan Valsartan 40, 80, and 160 mg bid tablets with placebo for sacubitril/valsartan
Target Patients	Pediatric patients from 1 month to < 18 years of age with heart failure due to systemic left ventricle systolic dysfunction	Patients with chronic heart failure with preserved ejection fraction
Read-out Milestone(s)	2022; (Analysis of 110 pts from Part 2 formed the basis for pediatric submission in Apr-2019 and approval by the US FDA in Oct-2019 for the treatment of symptomatic HF with systemic left ventricular systolic dysfunction in children aged 1 year and older)	2023
Publication	TBD	TBD

Entresto® - Angiotensin receptor/neprilysin inhibitor

Study	NCT03785405 (CLCZ696B2319E1 - extension study)
Indication	Heart failure in pediatric patients
Phase	Phase 3
Patients	240
Primary Outcome Measures	Number of participants with Adverse Events (AEs) and Serious Adverse Events (SAEs)
Arms Intervention	Single arm, open label sacubitril/valsartan (pediatric formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid))
Target Patients	Pediatric patients with heart failure due to systemic left ventricle systolic dysfunction who have completed study CLCZ696B2319
Read-out Milestone(s)	2023
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT03705234 ORION-4 (CKJX839B12301)
Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 3
Patients	~15000
Primary Outcome Measures	A composite of major adverse cardiovascular events, defined as: Coronary heart disease (CHD) death; Myocardial infarction; Fatal or non-fatal ischaemic stroke; or Urgent coronary revascularization procedure
Arms Intervention	Arm 1: every 6 month treatment Inclisiran sodium 300mg (given by subcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years Arm 2: matching placebo (given by subcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years.
Target Patients	Patient population with mean baseline LDL-C >= 100mg/dL
Read-out Milestone(s)	2026
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT03060577 ORION-3 (CKJX839A12201E1)	NCT03814187 ORION-8 (CKJX839A12305B)
Indication	Hypercholesterolemia inc. Atherosclerotic Cardiovascular Disease (ASCVD) and ASCVD risk equivalents Heterozygous Familial Hypercholesterolaemia (HeFH)	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 2	Phase 3
Patients	490	2991
Primary Outcome Measures	LDL-C reduction at Day 210 for Group 1 subjects Changes in other lipids and lipoproteins and reduction of LDL-C of more than 50% for patients that are above LDL-C goal ; longer term exposure and safety.	Proportion of subjects achieving pre specified low density lipoprotein cholesterol (LDL-C) targets at end of study Safety and tolerability profile of long term use of inclisiran
Arms Intervention	Group 1 - inclisiran sodium 300mg sc on Day 1 and every 180 days thereafter for up to 4 years. Group 2- Evolocumab 140mg s.c. injection on Day 1 and every 2 weeks until Day 336, followed by inclisiran sodium 300mg on Day 360, Day 450 and then every 6 months for a planned duration of 4 years.	Inclisiran sodium 300mg on day 1 (placebo patients entered into study from ORION 9, 10 & 11) or placebo on Day 1 (inclisiran patients entered into study from ORION 9, 10 & 11) then inclisiran sodium 300mg on Day 90 and every 6 months for a planned duration of 3 years
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 3, 9, 10 & 11 studies)
Read-out Milestone(s)	2021 (actual)	2023
Publication	TBD	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT03851705 ORION-5 (CKJX839A12302)	NCT04652726 ORION-16 (CKJX839C12301)
Indication	Hypercholesterolemia inc. Homozygous Familial Hypercholesterolemia (HoFH)	Hyperlipidemia, pediatrics
Phase	Phase 3	Phase 3
Patients	56	150
Primary Outcome Measures	LDL-C reduction at Day 150 Changes in PCSK9, other lipids and lipoproteins	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to Day 330
Arms Intervention	Part 1: inclisiran sodium 300mg on Day 1 and Day 90 or placebo on Day 1 and Day 90 Part 2: inclisiran sodium 300mg on Day 180 for patients who were randomized to the placebo group only, inclisiran sodium 300mg on Day 270 and then every 6 months for a planned duration of 2 years for all patients	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630; Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.
Target Patients	Patients with HoFH with background statin +/- ezetimibe therapy	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milestone(s)	Primary: Q3-2020 (actual); Final: H2-2021	2024
Publication	TBD	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT04659863 ORION-13 (CKJX839C12302)	NCT05030428 VICTORION-2P (CKJX839B12302)
Indication	Hyperlipidemia, pediatrics	CVRR
Phase	Phase 3	Phase 3
Patients	15	15000
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330	1. Time to First Occurrence of 3P-MACE (3-Point Major Adverse Cardiovascular Events)
Arms Intervention	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630;Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.	Arm 1: Experimental Inclisiran sodium, Subcutaneous injection Arm 2: Placebo Comparator, Placebo Subcutaneous injection
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)	Participants with established cardiovascular disease (CVD)
Read-out Milestone(s)	2024	2027
Publication	TBD	TBD

iptacopan - CFB inhibitor

Study	NCT04817618 APPEAR-C3G (CLNP023B12301)	NCT03955445 (CLNP023B12001B)
Indication	C3 glomerulopathy	C3 glomerulopathy (C3G)
Phase	Phase 3	Phase 2
Patients	68	27
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)	Characterize the effect of LNP023 treatment on a composite renal response endpoint at 9 months (1. a stable or improved eGFR and, 2. a reduction in proteinuria and 3. an increase in C3 compared to the CLNP023X2202 baseline visit)
Arms Intervention	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.	Open-label LNP023 200mg bid
Target Patients	Patients with native C3G	Patients with C3 glomerulopathy
Read-out Milestone(s)	2023	2025
Publication	TBD	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory

iptacopan - CFB inhibitor

Study	NCT04154787 (CLNP023D12201)	NCT04578834 APPLAUSE-IgAN (CLNP023A2301)
Indication	Idiopathic membranous nephropathy (iMN)	IgA nephropathy
Phase	Phase 2	Phase 3
Patients	72	450
Primary Outcome Measures	Change from baseline of UPCR derived from 24hr urine collections at Baseline and Week 24	Ratio to baseline in urine protein to creatinine ratio (sampled from 24h urine collection) at 9 months Annualized total estimated Glomerular Filtration Rate (eGFR) slope estimated over 24 months
Arms Intervention	LNP023 low dose LNP023 high dose Rituximab	Arm 1 - LNP023 200mg BID Arm 2 - Placebo BID
Target Patients	Patients with biopsy proven iMN who are at high risk of disease progression defined on the basis of antibody anti-PLA2R titre and proteinuria	Primary IgA Nephropathy patients
Read-out Milestone(s)	2023	2023 (primary endpoint for US initial submission, 9 months UPCR)2025 (24 months)
Publication	TBD	Perkovic et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: Study Design Wong et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: IPTACOPAN (LNP023): A NOVEL ORAL COMPLEMENT ALTERNATIVE PATHWAY FACTOR B INHIBITOR SAFELY AND EFFECTIVELY STABILISES EGFR IN C3 GLOMERULOPATHY

pelacarsen - ASO targeting Lp(a)

Study	NCT04023552 Lp(a)HORIZON (CTQJ230A12301)
Indication	Cardiovascular risk reduction
Phase	Phase 3
Patients	7680
Primary Outcome Measures	Time to the first occurrence of MACE (cardiovascular death, non-fatal MI, non-fatal stroke and urgent coronary re-vascularization)
Arms Intervention	TQJ230 80 mg injected monthly subcutaneously or matched placebo
Target Patients	Patients with a history of Myocardial infarction or Ischemic Stroke, or a clinically significant symptomatic Peripheral Artery Disease, and Lp(a) >= 70 mg/dL
Read-out Milestone(s)	2025
Publication	TBD

Immunology, Hepatology & Dermatology

LNA043- ANGPTL3 agonist

Study	NCT03275064 (CLNA043X2202)	NCT04864392 ONWARDS (CLNA043A12202)
Indication	Knee osteoarthritis	Knee osteoarthritis
Phase	Phase 2	Phase 2
Patients	133	550
Primary Outcome Measures	Articular cartilage bi-layer collagen organisation evaluated with MRI and measured in milliseconds (ms) (Part A only) Number of patients with any adverse events, serious adverse events and death (Part A and Part B) Change in cartilage volume/thickness in the index region (Part B only)	Change from baseline in the cartilage thickness of the medial compartment of the knee as assessed by imaging
Arms Intervention	LNA043 40 mg Part B LNA043 20 mg Part B LNA043 20 mg Part A Placebo Part A Placebo Part B	LNA043 injection to the knee with dosing regimen A LNA043 injection to the knee with dosing regimen B LNA043 injection to the knee with dosing regimen C LNA043 injection to the knee with dosing regimen D Placebo injection to the knee
Target Patients	Patients with cartilage lesions of the knee (Part A) and knee osteoarthritis (Part B)	Patients with Symptomatic knee osteoarthritis
Read-out Milestone(s)	2022	Primary 2024
Publication	TBD	TBD

Cosentyx® - IL-17A inhibitor

Study	NCT03031782 (CAIN457F2304)	NCT03259074 SURPASS (CAIN457K2340)
Indication	JPsA & ERA	JPsA & ERA
Phase	Phase 3	Phase 3
Patients	80	837
Primary Outcome Measures	Time to 33 flares	No radiographic structural progression as measured by modified Stoke Ankylosing Spondylitis Spine Score (mSASSS)
Arms Intervention	Secukinumab (pre-filled syringe) 75 mg Placebo	Secukinumab 150/300 mg Adalimumab biosimilar 40 mg
Target Patients	Juvenile idiopathic arthritis subtypes of psoriatic and enthesitis-related arthritis	Patients with active ankylosing spondylitis
Read-out Milestone(s)	H1-2021	2022
Publication	H2-2021	Study design manuscript published. Baraliakos et al. Clinical Drug Investigation (2020) 40:269-278.

Cosentyx® - IL-17A inhibitor

Study	NCT03713619 SUNSHINE (CAIN457M2301)	NCT03713632 SUNRISE (CAIN457M2302)
Indication	Hidradenitis Suppurativa (HS)	Hidradenitis Suppurativa (HS)
Phase	Phase 3	Phase 3
Patients	471	471
Primary Outcome Measures	Proportion of participants with Hidradenitis Suppurativa clinical response (HiSCR)	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)
Arms Intervention	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks Placebo (every 2 weeks) Placebo (every 4 weeks)	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks Placebo (every 2 weeks) Placebo (every 4 weeks)
Target Patients	Patients with moderate to severe Hidradenitis Suppurativa	Subjects with moderate to severe Hidradenitis Suppurativa
Read-out Milestone(s)	Primary (week 16): H2-2021; Final: 2022	Primary (week 16): H2-2021; Final: 2022
Publication	Study design SHSA 2020; Primary 2022	Study design SHSA 2020; Primary 2022

Cosentyx® - IL-17A inhibitor

Study	NCT03769168 (CAIN457F2304E1 - extension study)	NCT04156620 INVIGORATE-1 (CAIN457P12301)
Indication	Psoriatic arthritis	Axial spondyloarthritis
Phase	Phase 3	Phase 3
Patients	64	500
Primary Outcome Measures	Number of participants with JIA ACR30 response	The proportion of subjects achieving an ASAS40 (Assessment of SpondyloArthritis International Society criteria) response
Arms Intervention	Secukinumab 75 mg/0.5 ml Secukinumab 150 mg/1.0 ml	Secukinumab intravenous (i.v.) regimen Placebo intravenous (i.v.) regimen
Target Patients	Patients with juvenile idiopathic arthritis subtypes of juvenile psoriatic arthritis and enthesitis related arthritis	Patients with active axial spondyloarthritis
Read-out Milestone(s)	2025	Primary (week 16): 2022; Final: 2023
Publication	TBD	2023

Cosentyx® - IL-17A inhibitor

Study	NCT04179175 (CAIN457M2301E1)	NCT04181762 SELUNE (CAIN457Q12301)
Indication	Hidradenitis Suppurativa (HS)	Lupus Nephritis
Phase	Phase 3	Phase 3
Patients	745	460
Primary Outcome Measures	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)	Proportion of subjects achieving protocol-defined CRR
Arms Intervention	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with moderate to severe hidradenitis suppurativa completing either of the core trials AIN457M2301 (NCT 0313632) or AIN567M2302 (NCT03713619)	Patients with active lupus nephritis (ISN/RPS Class III or IV, with or without co-existing class V features)
Read-out Milestone(s)	2025	2026
Publication	Study design SHSA 2020	2026

Cosentyx® - IL-17A inhibitor

Study	NCT04209205 INVIGORATE-2 (CAIN457P12302)	NCT04300296 PRELUDE (CAIN457S12201)
Indication	Psoriatic Arthritis (PsA)	Lichen Planus
Phase	Phase 3	Phase 2
Patients	380	108
Primary Outcome Measures	The proportion of subjects achieving American College of Rheumatology 50 (ACR50) response criteria	Proportion of patients achieving Investigator's Global Assessment (IGA 0/1) score at 16 weeks +30% delta vs placebo
Arms Intervention	Secukinumab intravenous (i.v.) regimen Placebo intravenous (i.v.) regimen	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with active psoriatic arthritis (PsA) despite current or previous NSAID, DMARD and/or anti-TNF therapy	Adult patients with biopsy-proven lichen planus not adequately controlled by topical therapies
Read-out Milestone(s)	H2-2021 (Actual)	2022
Publication	2023	TBD

Cosentyx® - IL-17A inhibitor

Study	NCT04930094 (CAIN457R12301)
Indication	Giant cell arteritis
Phase	Phase 3
Patients	240
Primary Outcome Measures	Number of participants with sustained remission
Arms Intervention	Experimental: Secukinumab 300 mg Placebo Comparator: Placebo
Target Patients	Patients with Giant Cell Arteritis (GCA)
Read-out Milestone(s)	Primary 2024 Final 2025
Publication	TBD

ianalumab - BAFF-R inhibitor

Study	NCT03217422 AMBER (CVAY736B2201)
Indication	Autoimmune hepatitis
Phase	Phase 2
Patients	80
Primary Outcome Measures	Alanine aminotransferase (ALT) normalization
Arms Intervention	VAY736 Placebo control with conversion to active VAY736
Target Patients	Autoimmune hepatitis patients with incomplete response or intolerant to standard treatment of care
Read-out Milestone(s)	2026
Publication	TBD

iscalimab - CD40 inhibitor

Study	NCT03781414 CONTRAIL I (CCFZ533A2202)	NCT03905525 TWINSS (CCFZ533B2201)
Indication	Liver transplantation	Sjögren's syndrome
Phase	Phase 2	Phase 2
Patients	128	260
Primary Outcome Measures	Proportion of patients with composite event (BPAR, Graft Loss or Death) over 12 months	Change in EULAR Sjögren's syndrome Disease Activity Index (ESSDAI) score and EULAR Sjögren's syndrome Patient Reported Index (ESSPRI) score
Arms Intervention	Control/Standard of Care: TAC + MMF + Corticosteroids CFZ533 dose A + MMF + Corticosteroids CFZ533 dose B + MMF + Corticosteroids	Three dose arms of CFZ533 Placebo
Target Patients	Liver transplant recipients	Patients with Sjögren's syndrome
Read-out Milestone(s)	2023	2022
Publication	2023	2022

iscalimab - CD40 inhibitor

Study	**NCT04541589 TWINSS Extn (CFZ533B2201E1)**
Indication	Sjögren's syndrome
Phase	Phase 2
Patients	
Primary Outcome Measures	Incidence of Treatment-emergent AEs (TEAEs) Change in laboratory evaluations for hematology from baseline to each study visit Change in laboratory evaluations for serum chemistry from baseline to each study visit Change in vital sign measurements from baseline for each post-baseline visit
Arms Intervention	Arm 1 - Iscalimab Dose 1 s.c. Q2W Arm 2 - Iscalimab Dose 2 s.c. Q2W and Placebo
Target Patients	Patients with Sjögren's Syndrome, who participated in the TWINSS core study, CCFZ533B2201(NCT03905525)
Read-out Milestone(s)	Primary completion date: 2024
Publication	

ligelizumab - IgE inhibitor

Study	NCT03580369 Pearl 1 (CQGE031C2302)	NCT03580356 Pearl 2 (CQGE031C2303)
Indication	Chronic spontaneous urticaria	Chronic spontaneous urticarial / Chronic idiopathic urticaria?
Phase	Phase 3	Phase 3
Patients	1050	1079
Primary Outcome Measures	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12
Arms Intervention	Ligelizumab dose A q4w for 52 weeks Ligelizumab dose B q4w for 52 weeks Omalizumab 300 mg q4w for 52 weeks Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52	Ligelizumab dose A q4w for 52 weeks Ligelizumab dose B q4w for 52 weeks Omalizumab 300 mg q4w for 52 weeks Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52
Target Patients	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines
Read-out Milestone(s)	H2-2021 (actual)	H2-2021 (actual)
Publication	Past publications: Study design presented at UCARE 2018 Manuscripts - Primary results. PEARL1/2 pooled data. NEJM or Lancet. H2-2022 - H1-2023 (Dec 2022 or Jan 2023) Congress publications - EADV 2022: Late breaking abstract on primary results (efficacy, safety). H2-2022 as a first publication in Europe - ACAAI 2022: Primary results (efficacy, safety). H2-2022 as a first publication in the USA - AAAAI 2023: secondary results. H1-2023 - AAD 2023: secondary results. H1-2023	Past publications: Study design presented at UCARE 2018 Manuscripts - Primary results. PEARL1/2 pooled data. NEJM or Lancet. H2-2022 - H1-2023 (Dec 2022 or Jan 2023) Congress publications - EADV 2022: Late breaking abstract on primary results (efficacy, safety). H2-2022 as a first publication in Europe - ACAAI 2022: Primary results (efficacy, safety). H2-2022 as a first publication in the USA - AAAAI 2023: secondary results. H1-2023 - AAD 2023: secondary results. H1-2023

ligelizumab - IgE inhibitor

Study	**NCT04210843 (CQGE031C2302E1)**
Indication	Chronic spontaneous urticaria
Phase	Phase 3
Patients	1520
Primary Outcome Measures	The proportion of subjects with well-controlled disease (UAS7 ? 6) at week 12
Arms Intervention	Ligelizumab Dose 1 and 3 Ligelizumab Dose 2 and 3
Target Patients	Patients who completed studies CQGE031C2302, CQGE031C2303, CQGE031C2202 or CQGE031C1301
Read-out Milestone(s)	2026
Publication	Study design presented at 2020 EAACI

ligelizumab - IgE inhibitor

Study	NCT05024058 PEARL-PROVOKE (CQGE031E12301)	NCT04984876 PEANUT (CQGE031G12301)
Indication	CINDU	Food allergy
Phase	Phase 3	Phase 3
Patients	438	486
Primary Outcome Measures	1. Change from baseline in Total Fric Score in participants with symptomatic dermographism	1. Proportion of participants who can tolerate a single dose of ≥ 600 mg (1044 mg cumulative tolerated dose) of peanut protein without dose-limiting symptoms at Week 12
Arms Intervention	Arm 1: Experimental Ligelizumab low dose, symptomatic dermographism group Arm 2: Experimental Ligelizumab high dose, symptomatic dermographism Arm 3: Placebo Comparator. Placebo SC q4W, symptomatic dermographism Arm 4: Experimental Ligelizumab low dose, cold urticaria Arm 5: Experimental Ligelizumab high dose, cold urticaria Arm 6: Placebo Comparator: Placebo SC q4w, cold urticaria Arm 7: Experimental Ligelizumab high dose, cholinergic urticaria Arm 8: Placebo Comparator: Placebo SC q4w, cholinergic urticaria	Arm 1: Experimental igelizumab 240 mg subcutaneous injection for 52 weeks Arm 2: Experimental ligelizumab 120 mg subcutaneous injection for 52 weeks Arm 3: Experimental Placebo 8 weeks and ligelizumab 120 mg Arm 4: Placebo subcutaneous injection for first 8 weeks and ligelizumab 120 mg subcutaneous injection for 44 weeks Arm 5: Experimental Placebo 16 weeks and ligelizumab 120 mg/240 mg subcutaneous injection for 36 weeks Arm 6: Experimental Placebo 8 weeks and ligelizumab 240 mg subcutaneous injection for 44 weeks
Target Patients	Adolescents and adults with chronic inducible urticaria who remain symptomatic despite treatment with H1- Antihistamines	Participants with a medically confirmed diagnosis of IgE-mediated peanut allergy
Read-out Milestone(s)	2024	2025
Publication	TBD	

remibrutinib - BTK inhibitor

Study	NCT04109313 (CLOU064A2201E1)
Indication	Chronic spontaneous urticaria (CSU)
Phase	Phase 2
Patients	250
Primary Outcome Measures	Long-term safety and tolerability
Arms Intervention	Selected dose of LOU064 taken orally twice a day (morning and evening) from day 1 to week 52
Target Patients	Patients with CSU who have participated in preceding studies with LOU064
Read-out Milestone(s)	2022
Publication	TBD

remibrutinib - BTK inhibitor

Study	NCT05030311 REMIX-1 (CLOU064A2301)	NCT05032157 REMIX-2 (CLOU064A2302)
Indication	Chronic spontaneous urticaria (CSU)	Chronic spontaneous urticaria (CSU)
Phase	Phase 3	Phase 3
Patients	450	450
Primary Outcome Measures	1. Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint) 2. Absolute change in ISS7 and absolute change in HSS7 (Scenario 2 with ISS7 and HSS7 as co-primary efficacy endpoints)	1. Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint) 2. Absolute change in ISS7 an absolute change in HSS7 (Scenario 2 with ISS7 and HSS7 as co-primary efficacy endpoints)
Arms Intervention	Arm 1: LOU064 (blinded) LOU064 (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2). Arm 2: LOU064 placebo (blinded) LOU064 placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2)	Arm 1: LOU064 (blinded) LOU064A (blinded) taken orally b.i.d. for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks. Randomised in 2:1 ratio (active vs placebo) Arm 2: LOU064 placebo (blinded) LOU064A placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks. Randomised in 2:1 ratio (active vs placebo)
Target Patients	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo
Read-out Milestone(s)	2024	2024
Publication	TBD	TBD

tropifexor, licogliflozin - FXR agonist and SGLT 1/2 inhibitor

Study	NCT04065841 ELIVATE (CLJN452D12201C)
Indication	Non-alcoholic steatohepatitis (NASH)
Phase	Phase 2
Patients	380
Primary Outcome Measures	Proportion of patients with resolution of NASH and no worsening of fibrosis OR improvement in fibrosis by at least one stage without worsening of NASH at Week 48 compared with baseline
Arms Intervention	Arm A: combination therapytropifexor + licogliflozin Arm B: tropifexor monotherapytropifexor + licogliflozin placebo Arm C: licogliflozin monotherapylicogliflozin + tropifexor placebo Arm D: licogliflozin placebo + tropifexor placebo
Target Patients	Adult patients with biopsy based non-alcoholic steatohepatitis (NASH) and liver fibrosis
Read-out Milestone(s)	2023
Publication	2023

Neuroscience

MIJ821- NR2B negative allosteric modulator (NAM)

Study	NCT04722666 (CMIJ821A12201)
Indication	Acute depression
Phase	Phase 2
Patients	195
Primary Outcome Measures	Change from baseline to 24 hours in the total score of the Montgomery Åsberg Depression Rating Scale (MADRS)
Arms Intervention	MIJ821 (mg/kg) very low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1 or 0.9% sodium chloride
Target Patients	Participants who have suicidal ideation with intent
Read-out Milestone(s)	2023
Publication	TBD

Aimovig® - CGRP receptor antagonist

Study	NCT03867201 DRAGON (CAMG334A2304)
Indication	Migraine
Phase	Phase 3
Patients	550
Primary Outcome Measures	Change from baseline in monthly migraine days during the last 4 weeks of the 12-week treatment period
Arms Intervention	Subcutaneous injection of AMG334 (erenumab) 70 mg Subcutaneous injection of placebo
Target Patients	Adult chronic migraine patients
Read-out Milestone(s)	Double-blind FIR for 100% of pts 2021; Q4 2021(actual) Extension (open-label): 2024
Publication	Planned in H2-2022 for double-blind phase and H1-2025 for open-label extension phase

LMI070 - mRNA splicing modulator

Study	**NCT05111249 VIBRANT-HD (CLMI070C12203)**
Indication	Huntington`s disease
Phase	Phase 2
Patients	75
Primary Outcome Measures	1. Reduction (%) of mHTT protein in cerebrospinal fluid (CSF) 2. Number of treatment emergent adverse events and serious adverse events
Arms Intervention	Arm 1: Experimental; Branaplam 56 mg oral solution once weekly Arm 2: Experimental; Branaplam 112 mg oral solution once weekly Arm 3: Experimental; (C) Branaplam 154 mg oral solution once weekly, OR (X) Branaplam 84 mg oral solution once weekly OR (Y) Branaplam 28 mg oral solution once weekly Arm 4: Placebo; Matching placebo oral solution once weekly
Target Patients	Participants with early manifest Huntington's Disease
Read-out Milestone(s)	2025
Publication	TBD

Kesimpta® - CD20 antagonist

Study	NCT03650114 ALITHIOS (COMB157G2399)
Indication	Multiple Sclerosis
Phase	Phase 3
Patients	2010
Primary Outcome Measures	Evaluate the long-term safety and tolerability of ofatumumab 20 mg subcutaneous (sc) once every 4 (q4) weeks in subjects with RMS from the first dose of ofatumumab
Arms Intervention	Ofatumumab 20 mg every 4 weeks
Target Patients	Patients with relapsing MS
Read-out Milestone(s)	2028
Publication	TBD

Mayzent® - S1P1,5 receptor modulator

Study	NCT04926818 NEOS (CBAF312D2301)
Indication	Multiple sclerosis, pediatrics
Phase	Phase 3
Patients	180
Primary Outcome Measures	Annualized relapse rate (ARR) in target pediatric participants
Arms Intervention	Arm 1: Experimental ofatumumab - 20 mg injection/ placebo Arm 2: Experimental siponimod - 0.5 mg, 1 mg or 2 mg/ placebo Arm 3: Active Comparator fingolimod - 0.5 mg or 0.25 mg/ placebo
Target Patients	Children/adolescent patients aged 10-17 years old with Multiple Sclerosis (MS). he targeted enrollment is 180 participants with multiple sclerosis which will include at least 5 participants with body weight (BW) ?40 kg and at least 5 participants with age 10 to 12 years in each of the ofatumumab and siponimod arms. There is a minimum 6 month follow up period for all participants (core and extension). Total duration of the study could be up to 7 years.
Read-out Milestone(s)	2026
Publication	TBD

remibrutinib - BTK inhibitor

Study	NCT05147220 REMODEL-1 (CLOU064C12301)	NCT05156281 REMODEL-2 (CLOU064C12302)
Indication	Multiple sclerosis	Multiple sclerosis
Phase	Phase 3	Phase 3
Patients	800	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses	Annualized relapse rate (ARR) of confirmed relapses
Arms Intervention	Arm 1: Experimental; Remibrutinib - Core (Remibrutinib tablet and matching placebo of teriflunomide capsule) Arm 2: Active Comparator; Teriflunomide - Core (Teriflunomide capsule and matching placebo remibrutinib tablet) Arm 3: Experimental; Remibrutinib - Extension (Participants on remibrutinib in Core will continue on remibrutinib tablet) Arm 4: Experimental; Remibrutinib - Extension (on teriflunomide in Core) (Participants on teriflunomide in Core will switch to remibrutinib tablet)	Arm 1: Experimental; Remibrutinib - Core Remibrutinib tablet and matching placebo of teriflunomide capsule Arm 2: Active Comparator; Teriflunomide - Core Teriflunomide capsule and matching placebo remibrutinib tablet Arm 3: Experimental: Remibrutinib - Extension Participants on remibrutinib in Core will continue on remibrutinib tablet Arm 4: Experimental: Remibrutinib - Extension (on teriflunomide in Core) Participants on teriflunomide in Core will switch to remibrutinib tablet
Target Patients	Patients with relapsing Multiple Sclerosis	Patients with relapsing Multiple Sclerosis
Read-out Milestone(s)	Estimated primary completion 2025 Estimated study completion 2029	Estimated primary completion 2025 Estimated study completion 2029
Publication	TBD	TBD

Zolgensma® - SMN1 gene replacement therapy

Study **NCT05089656 STEER (COAV101B12301)**

Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3
Patients	125
Primary Outcome Measures	1. Change from baseline in Hammersmith functional motor scale - Expanded (HFMSE) total score at the end of follow-up period 1 in treated patients compared to sham controls in the ≥ 2 to < 18 years age group
Arms Intervention	Arm 1: Experimental OAV101. Administered as a single, one-time intrathecal dose Arm 2: Sham Comparator: Sham control. A skin prick in the lumbar region without any medication.
Target Patients	Patients Type 2 Spinal Muscular Atrophy (SMA) who are ≥ 2 to < 18 years of age, treatment naive, sitting, and never ambulatory
Read-out Milestone(s)	2024
Publication	TBD

Ophthalmology

UNR844 - Reduction of disulfide bonds

Study	NCT04806503 READER (CUNR844A2022)
Indication	Presbyopia
Phase	Phase 2B
Patients	225
Primary Outcome Measures	Characterize the dose response relationship among UNR844 doses 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily after Month 3 of dosing. Change from baseline in Binocular distance-corrected near visual acuity at 40 cm at Month 3.
Arms Intervention	1:1 randomization - UNR844 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily for three months
Target Patients	Presbyopic participants aged 45 to 55 years
Read-out Milestone(s)	2022: Primary endpoint- when all patients have completed the 3 months treatment period 2023: Final analysis -Study completion (all patients have completed 9 months pots treatment period)
Publication	H1-2023

Beovu® - Anti-VEGF

Study	NCT03386474 (CRTH258A2301E1)	NCT04005352 TALON (CRTH258A2303)
Indication	Neovascular age-related macular degeneration (nAMD)	Neovascular Age-related Macular Degeneration (nAMD)
Phase	Phase 3	Phase 3B
Patients	150	
Primary Outcome Measures	Number of treatment-emergent adverse events	Average change in Best-corrected visual acuity Distribution of the last interval with no disease activity (in a Treat-to-Control regimen)
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Arm 1: Brolucizumab 6 mg intravitreal injection Arm 2: Aflibercept 2 mg intravitreal injection
Target Patients	Patients with neovascular age-related macular degeneration who have completed the CRTH258A2301 study	Patients with Neovascular Age-related Macular Degeneration (nAMD) who have not previously received anti-VEGF (vascular endothelial growth factor) treatment
Read-out Milestone(s)	2018 (actual)	2022
Publication	Manuscript submitted	TBD

Beovu® - Anti-VEGF

Study	NCT04047472 HOBBY (CRTH258A2307)
Indication	Macular degeneration
Phase	Phase 3
Patients	494
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA) at week 48
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Chinese patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2024
Publication	TBD

Beovu® - VEGF Inhibitor

Study	NCT03481634 KESTREL (CRTH258B2301)	NCT03481660 KITE (CRTH258B2302)
Indication	Diabetic eye disease	Diabetic eye disease
Phase	Phase 3	Phase 3
Patients	534	356
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA)	Change from baseline in best-corrected visual acuity (BCVA)
Arms Intervention	Brolucizumab (RTH258) 3 mg/50 µL Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2mg/50 uL	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema (DME)	Patients with visual impairment due to diabetic macular edema (DME)
Read-out Milestone(s)	Primary: Q4-2020 (actual); Final: Q4-2021	Primary: Q3-2020 (actual); Final: Q3-2021 (actual).
Publication	Brown et al., presented at ARVO May 2021Manuscript submission H2 2021 (Actual)	Brown et al., presented at ARVO May 2021Manuscript submission H2 2021 (Actual)

Beovu® - VEGF Inhibitor

Study	NCT03917472 KINGFISHER (CRTH258B2305)	NCT04058067 KINGLET (CRTH258B2304)
Indication	Diabetic macular edema	Diabetic macular edema
Phase	Phase 3	Phase 3
Patients	500	268
Primary Outcome Measures	Change in best-corrected visual acuity (BCVA) from baseline up to week 52	Change in best-corrected visual acuity (BCVA)
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema	Chinese patients with visual impairment due to diabetic macular edema
Read-out Milestone(s)	Q3-2021 (Actual)	2023
Publication	Publication planned for H1-2022	Publication planned for 2023

Beovu® - VEGF Inhibitor

Study	NCT04278417 (CRTH258D2301)
Indication	Diabetic retinopathy
Phase	Phase 3
Patients	706
Primary Outcome Measures	Change from Baseline in BCVA
Arms Intervention	Arm1: RTH258 (brolucizumab) 6 mg/50uL Arm2: Panretinal photocoagulation laser initial treatment followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Read-out Milestone(s)	2024
Publication	TBD

libvatrep - TRPV1 antagonist

Study	NCT04630158 SAHARA (CSAF312B12201)
Indication	Chronic ocular surface pain
Phase	Phase 2
Patients	150
Primary Outcome Measures	Change in mean pain severity Visual Analog Scale
Arms Intervention	Placebo Comparator: SAF312 Placebo. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 1. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 2. Randomized to a 1:1:1 topical eye drops, twice daily
Target Patients	Subjects with CICP persisting at least for 4 months after refractive surgery and chronicity confirmed during the observational period.
Read-out Milestone(s)	2023
Publication	2023

Respiratory & Allergy

CSJ117 - Inhaled TSLP inhibitor

Study	**NCT04410523 (CCSJ117A12201C)**
Indication	Asthma
Phase	Phase 2
Patients	625
Primary Outcome Measures	Pre-dose FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment. Average change from baseline in pre-dose FEV1 at week 8 & week 12
Arms Intervention	CSJ117 0.5mg CSJ117 1mg CSJ117 2 mg CSJ117 4 mg CSJ117 8 mg Placebo
Target Patients	Asthma patients on background medium or high ICS plus LABA therapy
Read-out Milestone(s)	2023
Publication	2023

icenticaftor - CFTR potentiator

Study	NCT04072887 (CQBW251B2201)
Indication	Chronic obstructive pulmonary disease (COPD)
Phase	Phase 2
Patients	956
Primary Outcome Measures	Trough FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment
Arms Intervention	QBW251 450 mg QBW251 300 mg QBW251 150 mg QBW251 75 mg QBW251 25 mg Placebo
Target Patients	COPD patients on background triple inhaled therapy (LABA / LAMA / ICS)
Read-out Milestone(s)	2022
Publication	Primary publications planned for 2022

Oncology: Solid Tumors

alpelisib - PI3K-alpha inhibitor

Study	NCT04589650 EPIK-P2 (CBYL719F12201)
Indication	PIK3CA-related overgrowth spectrum
Phase	Phase 2
Patients	150
Primary Outcome Measures	Proportion of participants with a response at Week 24
Arms Intervention	Arm 1: alpelisib vs. Arm 2: placebo during the 16 first weeks, for each cohort (adult, pediatric), with placebo patients switching to alpelisib thereafter.
Target Patients	Pediatric and adult participants with PIK3CA-related overgrowth spectrum (PROS)
Read-out Milestone(s)	Primary Analysis: 2023
Publication	NA

canakinumab - IL-1beta inhibitor

Study	NCT03631199 CANOPY-1 (CACZ885U2301)
Indication	1st Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3
Patients	627
Primary Outcome Measures	Safety run-in part: Incidence of dose limiting toxicities Double-blind, randomized, placebo-controlled part: Progression free survival (PFS) Overall survival (OS)
Arms Intervention	Canakinumab or matching placebo in combination with pembrolizumab and platinum-based doublet chemotherapy
Target Patients	Patients with: Histologically confirmed Stage IIIB, IV NSCLC with no prior systemic anticancer therapy Squamous and non-squamous NSCLC No EGFR mutation and ALK rearrangement
Read-out Milestone(s)	H2-2021
Publication	Johnson B et al. Presented at AACR-NCI-EORTC 2019 (safety run-in) Planned abstract submission to AACR 2022

canakinumab - IL-1beta inhibitor

Study	NCT03447769 CANOPY-A (CACZ885T2301)
Indication	Adjuvant NSCLC
Phase	Phase 3
Patients	1500
Primary Outcome Measures	Disease free survival (primary), overall survival (key secondary)
Arms Intervention	Canakinumab 200mg q3w sc for 18 cycles Placebo q3w sc for 18 cycles
Target Patients	Patients with: High-risk NSCLC (AJCC/UICC v.8 stage II-IIIA and IIIB (T>5cm N2)) after complete resection and standard of care adjuvant cisplatin-based chemotherapy All histologies
Read-out Milestone(s)	2022
Publication	TBD

NIS793 - TGFβ1 inhibitor

Study	**NCT04935359 (CNIS793B12301)**
Indication	Pancreatic cancer, 1st line
Phase	Phase 3
Patients	490
Primary Outcome Measures	Safety run-in part: Percentage of participants with dose limiting toxicities (DLTs) during the first cycle (4 weeks) of treatment Randomized part: Overall survival (OS)
Arms Intervention	Arm 1: Experimental: Safety run-in part: NIS793+gemcitabine+nab-paclitaxel In the safety run-in part, participants will receive a combination of NIS793, gemcitabine and nab-paclitaxel Arm 2: Experimental: Randomized part: NIS793+gemcitabine+nab-paclitaxel Participants will receive a combination of NIS793, gemcitabine and nab-paclitaxel Arm 3: Placebo Comparator: Randomized part: placebo+gemcitabine+nab-paclitaxel Participants will receive a combination of placebo, gemcitabine and nab-paclitaxel
Target Patients	Patients with Metastatic Pancreatic Ductal Adenocarcinoma (mPDAC), first line treatment
Read-out Milestone(s)	Primary 2025
Publication	TBD

TNO155 - SHP2 inhibitor

Study	NCT03114319 (CTNO155X2101)	NCT04000529 (CTNO155B12101)
Indication	Solid tumors (single agent)	Solid tumors (combo)
Phase	Phase 1	Phase 1
Patients	255	126
Primary Outcome Measures	Number of participants with adverse events Number of participants with dose limiting toxicities	Incidence of dose limiting toxicities (DLTs) during the first cycle of combination treatment during the dose escalation part Incidence and severity of adverse events (AEs) and serious adverse events (SAEs) as per CTCAE v5.0, by treatment Dose tolerability
Arms Intervention	Drug: TNO155 Drug: TNO155 in combination with EGF816 (nazartinib)	TNO155 and Spartalizumab (PDR001) TNO155 and Ribociclib (LEE011)
Target Patients	Adult patients with advanced solid tumors in selected indications	Patients with advanced malignancies
Read-out Milestone(s)	2023	2022
Publication	TBD	TBD

^{177}Lu-PSMA-617 - Radioligand therapy target PSMA

Study	NCT04689828 PSMAfore (CAAA617B12302)	NCT04720157 PSMAddition (CAAA617C12301)
Indication	Metastatic castration-resistant prostate cancer, pre-taxane	Metastatic hormone sensitive prostate cancer
Phase	Phase 3	Phase 3
Patients	450	1126
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: Participants will receive 7.4 GBq (200 mCi) +/- 10% 177Lu-PSMA-617 once every 6 weeks for 6 cycles. Best supportive care, including ADT may be used Arm 2: For participants randomized to the ARDT arm, the change of ARDT treatment will be administered per the physician's orders. Best supportive care, including ADT may be used	Arm 1: 177Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) 177Lu-PSMA-617, once every 6 weeks (+/- 1 week) for a planned 6 cycles, in addition to the Standard of Care (SOC); ARDT +ADT is considered as SOC and treatment will be administered per the physician's order Arm 2: For participants randomized to Standard of Care arm, ARDT +ADT is considered as SOC and treatment will be administered per the physician's order
Target Patients	mCRPC patients that were previously treated with an alternate ARDT and not exposed to a taxane-containing regimen in the CRPC or mHSPC settings	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)
Read-out Milestone(s)	Primary Analysis: 2022 Final Analysis: 2025	Primary Analysis: 2024
Publication	TBD	TBD

Kisqali® - CDK4/6 inhibitor

Study	NCT03701334 NATALEE (CLEE011O12301C)
Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	5101
Primary Outcome Measures	Invasive Disease-Free Survival for using STEEP criteria (Standardized Definitions for Efficacy End Points in adjuvant breast cancer trials)
Arms Intervention	Ribociclib + endocrine therapy Endocrine therapy
Target Patients	Pre and postmenopausal women and men with HR-positive, HER2-negative EBC, after adequate surgical resection, who are eligible for adjuvant endocrine therapy
Read-out Milestone(s)	2022
Publication	TBD

Piqray® - PI3K-alpha inhibitor

Study	NCT04208178 EPIK-B2 (CBYL719G12301)	NCT04251533 EPIK-B3 (CBYL719H12301)
Indication	HER-2 positive breast cancer	Triple negative breast cancer
Phase	Phase 3	Phase 3
Patients	548	566
Primary Outcome Measures	Progression-free survival (PFS)	Progression-free Survival (PFS) for patients with PIK3CA mutant status
Arms Intervention	Alpelisib + trastuzumab + pertuzumab Trastuzumab + pertuzumab	Alpelisib 300 mg + nab-paclitaxel 100 mg/m² Placebo + nab-paclitaxel 100 mg/m²
Target Patients	Patients with HER2-positive advanced breast cancer with a PIK3CA mutation	Patients with advanced triple negative breast cancer with either Phosphoinositide-3-kinase Catalytic Subunit Alpha (PIK3CA) mutation or Phosphatase and Tensin Homolog Protein (PTEN) loss without PIK3CA mutation
Read-out Milestone(s)	2025	2023
Publication	TBD	TBD

Piqray® - PI3K-alpha inhibitor

Study	NCT04729387 EPIK-O (CBYL719K12301)
Indication	Ovarian Cancer
Phase	Phase 3
Patients	358
Primary Outcome Measures	Progression Free Survival (PFS) based on Blinded Independent Review Committee (BIRC) assessment using RECIST 1.1 criteria
Arms Intervention	Arm 1 Experimental: Alpelisib+olaparib: Alpelisib 200 mg orally once daily and olaparib 200 mg orally twice daily on a continuous dosing schedule Arm 2 Active Comparator: Paclitaxel or PLD. Investigator's choice of one of 2 single agent cytotoxic chemotherapies: Paclitaxel 80 mg/m2 intravenously weekly or Pegylated liposomal Doxorubicin (PLD) 40-50 mg/m2 (physician discretion) intravenously every 28 days.
Target Patients	Patients with platinum resistant or refractory high-grade serous ovarian cancer, with no germline BRCA mutation detected
Read-out Milestone(s)	2023
Publication	TBD

Tabrecta® - MET inhibitor

Study	NCT04427072 (CINC280A2301)	NCT04816214 GEOMETRY-E (CINC280L12301)
Indication	Non-small cell lung cancer	Non-small cell lung cancer
Phase	Phase 3	Phase 3
Patients	90	245
Primary Outcome Measures	Progression free survival (PFS) per blinded independent review committee (BIRC) using RECIST v1.1	Run-in part: Incidence of dose limiting toxicities (DLTs) Randomized part: Progression free survival (PFS)
Arms Intervention	Arm 1: 400mg of capmatinib tablets administered orally twice daily Arm 2: Docetaxel 75 mg/m2 by intravenous infusion every 21 days	Arm 1: Experimental: Combination of capmatinib + osimertinib (run-in part) Arm 2: Experimental: Combination of capmatinib + osimertinib (randomized part) Arm 3: Active Comparator: platinum + pemetrexed based doublet chemotherapy
Target Patients	Previously Treated Patients With EGFR wt, ALK Negative, Locally Advanced or Metastatic (Stage IIIB/IIIC or IV) NSCLC Harboring MET Exon 14 Skipping Mutation (MET?ex14).	Adult subjects with Non-small Cell Lung cancers as second line therapy
Read-out Milestone(s)	Primary 2022 Final: 2024	Primary: 2025 Final: 2027
Publication	TBD	TBD

Tafinlar + Mekinist® - BRAF inhibitor and MEK inhibitor

Study	**NCT04940052 (CDRB436J12301)**
Indication	Thyroid cancer
Phase	Phase 3
Patients	150
Primary Outcome Measures	Progression Free Survival
Arms Intervention	Arm 1: Experimental: Dabrafenib plus trametinib Participants will be treated with dabrafenib twice daily and trametinib once daily Arm 2: Placebo Comparator: Placebo dabrafenib plus placebo trametinib Participants will receive placebo dabrafenib twice daily and placebo trametinib once daily
Target Patients	Previously treated patients with locally advanced or metastatic, radio-active Iodine refractory BRAFV600E mutation-positive differentiated thyroid cancer
Read-out Milestone(s)	2024
Publication	TBD

Tafinlar + Mekinist® - BRAF inhibitor and MEK inhibitor

Study	NCT02684058 (CDRB436G2201)
Indication	BRAFV600 mutant gliomas
Phase	Phase 2
Patients	142
Primary Outcome Measures	Objective response rate
Arms Intervention	Dabrafenib + trametinib (dose based on age and weight)
Target Patients	Children and adolescent patients with BRAF V600 mutation positive relapsed or refractory high grade glioma (HGG) or BRAF V600 mutation positive low grade glioma (LGG)
Read-out Milestone(s)	Q4 2021 (actual)
Publication	TBD

Hematology

Adakveo® - P-selectin inhibitor

Study	NCT03814746 STAND (CSEG101A2301)
Indication	Prevention of Vaso-Occlusive Crises (VOC) in patients with Sickle Cell Disease (SCD)
Phase	Phase 3
Patients	240
Primary Outcome Measures	Rate of VOC events leading to healthcare visit
Arms Intervention	Crizanlizumab 5.0 mg/kg Crizanlizumab 7.5 mg/kg Placebo
Target Patients	Adolescent and adult SCD patients (12 years and older)
Read-out Milestone(s)	2022
Publication	TBD

Adakveo® - P-selectin inhibitor

Study	NCT03474965 SOLACE-Kids (CSEG101B2201)
Indication	Prevention of VOC in pediatric patients with SCD
Phase	Phase 2
Patients	100
Primary Outcome Measures	PK/PD and safety of SEG101 at 5 mg/kg
Arms Intervention	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide
Target Patients	Pediatric SCD patients with VOC
Read-out Milestone(s)	H2-2021 (pediatric patients >=12 year old) 2024 (pediatric patients <12 year old)
Publication	Abstract submission to ASH 2021

Jakavi® - JAK 1/2 inhibitor

Study	NCT03491215 REACH4 (CINC424F12201)	NCT03774082 REACH5 (CINC424G12201)
Indication	Acute graft versus host disease	Chronic graft versus host disease
Phase	Phase 2	Phase 2
Patients	45	45
Primary Outcome Measures	Measurement of PK parameters Overall Response Rate (ORR)	Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib	Ruxolitinib 5mg tablets / pediatric formulation
Target Patients	Pediatric patients with grade II-IV acute graft vs. host disease after allogeneic hematopoietic stem cell transplantation	Pediatric subjects with moderate and severe chronic Graft vs. Host disease after allogeneic stem cell transplantation
Read-out Milestone(s)	2023	2023
Publication	TBD	TBD

Jakavi® - JAK 1/2 inhibitor

Study	NCT04097821 ADORE (CINC424H12201)
Indication	Myelofibrosis
Phase	Phase 1/2
Patients	130
Primary Outcome Measures	Incidence of dose limiting toxicities within the first 2 cycles Response rate at the end of cycle 6
Arms Intervention	Ruxolitinib Ruxolitinib+Siremadlin Ruxolitinib+Crizanlizumab Ruxolitinib+MBG453 Ruxolitinib+LTT462 Ruxolitinib+NIS793
Target Patients	Patients with Myelofibrosis (MF)
Read-out Milestone(s)	2024
Publication	TBD

Kymriah® - CD19 CAR-T

Study	NCT03570892 BELINDA (CCTL019H2301)	NCT03876769 CASSIOPEIA (CCTL019G2201J)
Indication	2nd line Diffuse large B-cell lymphoma (DLBCL)	1st line high risk acute lymphoblastic leukemia (ALL)
Phase	Phase 3	Phase 2
Patients	318	160
Primary Outcome Measures	Event-free Survival (EFS)	Disease Free Survival (DFS)
Arms Intervention	Tisagenlecleucel versus standard of care	Single-arm study of tisagenlecleucel
Target Patients	Adult patients with aggressive B-cell Non-Hodgkin Lymphoma after failure of rituximab and anthracycline- containing frontline immunochemotherapy	Pediatric and young adult patients with 1st line high risk ALL
Read-out Milestone(s)	9 Jul 2021 (actual)	2025
Publication	Bishop et al at SITC 2019 Abstract submission TBD	TBD

Promacta® - Thrombopoetin receptor agonist

Study	NCT03025698 (CETB115E2201)	NCT03988608 (CETB115E2202)
Indication	Refractory or relapsed severe aplastic anemia	Refractory or relapsed severe aplastic anemia
Phase	Phase 2	Phase 2
Patients	51	20
Primary Outcome Measures	PK of eltrombopag at steady state in pediatric patients with SAA	Hematologic response rate rate up to 26 weeks of treatment
Arms Intervention	Eltrombopag 12.5, 25, 50, 75 mg FCT & 25 mg pFOS Arm A: relapsed/refractory SAA or recurrent AA following IST for SAA: hATG/cyclosporine + eltrombopag or cyclosporine + eltrombopag Arm B: previously untreated SAA: hATG/cyclosporine + eltrombopag	Eltrombopag 25 mg film-coated tablets
Target Patients	Pediatric patients from age 1 <18 years with relapsed/refractory SAA or recurrent AA after IST or previously untreated SAA	Chinese patients with refractory or relapsed severe aplastic anemia
Read-out Milestone(s)	Primary CSR: 2022 Final CSR: 2025	Primary CSR: 2022 Final CSR: 2025
Publication	TBD	TBD

Rydapt® - Multi-targeted kinase inhibitor

Study	NCT03591510 (CPKC412A2218)
Indication	Acute myeloid leukemia, pediatrics
Phase	Phase 2
Patients	20
Primary Outcome Measures	Occurrence of dose limiting toxicities Safety and Tolerability
Arms Intervention	Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Read-out Milestone(s)	2026
Publication	TBD

asciminib - BCR-ABL inhibitor

Study	**NCT04971226 ASC4FIRST (CABL001J12301)**
Indication	Chronic myeloid leukemia, 1st line
Phase	Phase 3
Patients	402
Primary Outcome Measures	Major Molecular Response (MMR) at week 48
Arms Intervention	Arm 1: asciminib 80 mg QD Arm 2: Investigator selected TKI including one of the below treatments: - Imatinib 400 mg QD - Nilotinib 300 mg BID - Dasatinib 100 mg QD - Bosutinib 400 mg QD
Target Patients	Patients with newly diagnosed philadelphia chromosome positive chronic myelogenous leukemia in chronic phase
Read-out Milestone(s)	2024
Publication	TBD

iptacopan - CFB inhibitor - HEM

Study	NCT03439839 (CLNP023X2201)	NCT03896152 (CLNP023X2204)
Indication	Paroxysmal nocturnal hemoglobinuria (PNH)	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 2	Phase 2
Patients	16	13
Primary Outcome Measures	Reduction of chronic hemolysis, based on LDH level at Week 13	Reduction of PNH associated hemolysis, based on percentage of patients with 60% reduction in LDH or LDH below upper limit of normal up to 12 weeks of treatment.
Arms Intervention	10 patients receiving LNP023 high dose daily over up to approximately 3 years 5 patients receiving LNP023 low dose daily over up to approximately 3 years	approximately 2 year Treatment with low LNP023 dose approximately 2 year Treatment with higher LNP023 dose
Target Patients	Patients with PNH, showing signs of active hemolysis despite treatment with SoC (defined as an antibody with anti C5 activity).	Patients with PNH, showing signs of active hemolysis, not treated with any other complement inhibitor less than 3 months prior to study start Day 1
Read-out Milestone(s)	Primary: Q2-2020 (actual) Extension: 2023	Primary: Q2-2020 (actual) Extension: 2022
Publication	Antonio M. Risitano, MD, PhD1 et al. Presented at EBMT 2020 congress Jan 2021Pubs: Addition of iptacopan, an oral factor B inhibitor, to eculizumab in patients with paroxysmal nocturnal haemoglobinuria and active haemolysis: an open-label, single-arm, phase 2, proof-of-concept trial, Risitano, Antonio M et al. The Lancet Haematology, Volume 8, Issue 5, e344 - e354	-Jang JH, et al. Presented at Korean Society of Hematology International Conference and 62nd Annual Meeting (ICKSH 2021) -Presented as an oral presentation (encore) at the European Haematology Association (EHA 2021) congress -Planned manuscript submission in Q3 2021

iptacopan - CFB inhibitor - HEM

Study	NCT04558918 APPLY-PNH (CLNP023C12302)	NCT04820530 APPOINT-PNH (CLNP023C12301)
Indication	Paroxysmal nocturnal haemoglobinuria	Paroxysmal nocturnal haemoglobinuria
Phase	Phase 3	Phase 3
Patients	91	40
Primary Outcome Measures	Percentage of participants achieving a sustained increase in hemoglobin levels of >= 2 g/dL in the absence of red blood cell transfusions Percentage of participants achieving sustained hemoglobin levels >= 12 g/dL in the absence of red blood cell transfusions	Proportion of participants achieving a sustained increase from baseline in hemoglobin levels of ? 2 g/dL assessed , in the absence of red blood cell transfusions
Arms Intervention	Arm 1: Drug: LNP023, taken orally b.i.d. dosage supplied: 200 mg dosage form: hard gelatin capsule Route of Administration: Oral Arm 2: Drug: Eculizumab, administered as intravenous infusion every 2 weeks as per the stable regimen, the maintenance dose is a fixed dose. Dosage supplied: 300 mg/30mL Dosage form: Concentrate solution for infusion Drug: Ravulizumab, administered as intravenous infusion every 8 weeks, the maintenance dose is based on body weight. Dosage Supplied: 300 mg/30mL Dosage f	Iptacopan (LNP023), taken orally b.i.d. (dosage supplied: 200mg)
Target Patients	Adult patients with PNH and residual anemia, despite treatment with an intravenous Anti-C5 antibody	PNH patients who are naive to complement inhibitor therapy, including anti-C5 antibody
Read-out Milestone(s)	Primary 2022	2023
Publication	Risitano AM, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)	Peffault de Latour R, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)

iptacopan - CFB inhibitor

Study	NCT04889430 APPELHUS (CLNP023F12301)
Indication	Atypical haemolytic uraemic syndrome
Phase	Phase 3
Patients	50
Primary Outcome Measures	Percentage of participants with complete TMA response without the use of PE/PI and anti-C5 antibody
Arms Intervention	Single arm open-label with 50 adult patients receiving 200mg oral twice daily doses of iptacopan
Target Patients	Adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody)
Read-out Milestone(s)	2024
Publication	TBD

sabatolimab - TIM3 antagonist

Study	NCT03946670 STIMULUS MDS-1 (CMBG453B12201)	NCT04150029 STIMULUS-AML1 (CMBG453C12201)
Indication	Myelodysplastic syndrome	Unfit acute myeloid leukaemia
Phase	Phase 2	Phase 2
Patients	120	86
Primary Outcome Measures	Complete Remission (CR) rate and Progression Free Survival (PFS)	Incidence of dose limiting toxicities (Safety run-in patients only) Percentage of subjects achieving complete remission (CR)
Arms Intervention	Experimental: Sabatolimab (MBG453) + hypomethylating agents Placebo comparator: Placebo + hypomethylating agents	Single arm safety and efficacy study of sabatolimab in combination with azacitidine and venetoclax
Target Patients	Adult subjects with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as per IPSS-R criteria	Newly diagnosed adult AML patients who are not suitable for treatment with intensive chemotherapy
Read-out Milestone(s)	2022-2023	2023
Publication	TBD	TBD

sabatolimab - TIM3 antagonist

Study	NCT04266301 STIMULUS-MDS2 (CMBG453B12301)
Indication	Myelodysplastic syndrome
Phase	Phase 3
Patients	500
Primary Outcome Measures	Overall survival
Arms Intervention	Sabatolimab 800 mg + azacitidine 75 mg/m2 Sabatolimab 800 mg + azacitidine 75 mg/m2 + placebo
Target Patients	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Read-out Milestone(s)	2023
Publication	TBD

Biosimilars

aflibercept - VEGF inhibitor

Study	NCT04864834 Mylight (CSOK583A12301)
Indication	Aflibercept BioS
Phase	Phase 3
Patients	460
Primary Outcome Measures	Best-corrected visual acuity (BCVA) will be assessed using the ETDRS testing charts at an initial distance of 4 meters. The change from baseline in BCVA in letters is defined as difference between BCVA score between week 8 and baseline
Arms Intervention	Arm 1 Biological: SOK583A1 (40 mg/mL) Arm 2 Biological: Eylea EU (40 mg/mL)
Target Patients	Patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2023
Publication	tbd

denosumab - anti RANKL mAb

Study	NCT03974100 (CGP24112301)
Indication	Denosumab BioS
Phase	Phase 3
Patients	522
Primary Outcome Measures	Percent change from baseline (%CfB) in lumbar spine Bone Mineral Density
Arms Intervention	GP2411 60 mg /mL subcutaneous injection every 6 months Prolia® 60 mg /mL subcutaneous injection every 6 months
Target Patients	Postmenopausal women with osteoporosis
Read-out Milestone(s)	2022
Publication	Study data publications expected for 2024 and beyond. The overall study design will be published at WCO and ECTS congresses 2020.

Global Health

artemether + lumefantrine

Study	NCT04300309 CALINA (CCOA566B2307)
Indication	Malaria, uncomplicated (<5kg patients)
Phase	Phase 3
Patients	
Primary Outcome Measures	Artemether Cmax
Arms Intervention	Experimental: artemether lumefantrine (2.5 mg:30 mg) artemether lumefantrine (2.5 mg:30 mg) bid over 3 days, from 1-4 tablets per dose
Target Patients	Infants and Neonates <5 kg body weight with acute uncomplicated plasmodium falciparum malaria
Read-out Milestone(s)	Primary outcome measure: 2023
Publication	TBD

ganaplacide - Imidazolopiperazines derivative

Study	NCT03167242 (CKAF156A2202)	NCT04546633 KALUMI (CKAF156A2203)
Indication	Malaria	Malaria, uncomplicated
Phase	Phase 2	Phase 2
Patients		
Primary Outcome Measures	PCR-corrected adequate clinical and parasitological response (ACPR)	PCR-corrected and uncorrected Adequate Clinical and Parasitological Response (ACPR)
Arms Intervention	KAF156 and LUM-SDF (different combinations) Coartem	KAF156 and LUM-SDF QD (once daily) for 2 days in fasted condition KAF156 and LUM-SDF QD (once daily) for 2 days in fed condition
Target Patients	Adults and children with uncomplicated Plasmodium falciparum malaria	Malaria patients 12 to < 18 years old with malaria caused by P. falciparum
Read-out Milestone(s)	H2-2021 (actual)	2024
Publication	No new publications	TBD

Abbreviations

aBC	Advanced breast cancer
AD	Atopic Dermatitis
Adj.	Adjuvant
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AMI	Acute myocardial infarction
AML	Acute myeloid leukemia
aNHL	Agressive non-Hodgkin's lymphoma
AS H2H	Ankylosing spondylitis head-to-head study versus adalimumab
BC	Breast cancer
C3G	C3 glomerulopathy
CCF	Congestive cardiac failure
CINDU	Chronic inducible urticaria
CLL	Chronic lymphocytic leukemia
CML	Chronic myeloid leukemia
CRC	Colorectal cancer
COPD	Chronic obstructive pulmonary disease
COSP	Chronic ocular surface pain
CRSwNP	Severe chronic rhinosinusitis with nasal polyps
CSU	Chronic spontaneous urticaria
CVRR-Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a)
CVRR-LDLC	Secondary prevention of cardiovascular events in patients with elevated levels of LDLC
DME	Diabetic macular edema
DLBCL	Diffuse large B-cell lymphoma refractory
ESCC	Esophageal squamous-cell carcinoma
FL	Follicular lymphoma
GCA	Giant cell arteritis
GVHD	Graft-versus-host disease
HCC	Hepatocellular carcinoma
HD	Huntington's disease
HFpEF	Chronic heart failure with preserved ejection fraction
HF-rEF	Chronic heart failure with reduced ejection fraction
HNSCC	Head and neck squamous cell carcinoma
HS	Hidradenitis suppurativa
IA	Interim analysis
IgAN	IgA nephropathy
iMN	Membranous nephropathy
IPF	Idiopathic pulmonary fibrosis
JIA	Juvenile idiopathic arthritis
jPsA/ERA	Juvenile psoriatic arthritis / enthesitis-related arthritis
LVEF	Left ventricular ejection fraction
mCRPC	Metastatic castration-resistant prostate cancer
MDR	Multi-drug resistant
MDS	Myelodysplastic syndrome
MS	Multiple sclerosis
NASH	Non-alcoholic steatohepatitis
nHCM	Non-obstructive hypertrophic cardiomyopathy
nr-axSpA	Non-radiographic axial spondyloarthritis
NSCLC	Non-small cell lung cancer
PEF	Preserved ejection fraction
PedPsO	Pediatric psoriasis
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
PROS	PIK3CA related overgrowth spectrum
RA	Rheumatoid arthritis
rMS	Relapsing multiple sclerosis
RVO	Retinal vein occlusion
SAA	Severe aplastic anemia
SLE	Systemic lupus erythematosus
SMA Type 1	Spinal muscular atrophy (IV formulation)
SMA Type 2/3	Spinal muscular atrophy (IT formulation)
SpA	Spondyloarthritis
SPMS	Secondary progressive multiple sclerosis
TNBC	Triple negative breast cancer
T1DM	Type 1 Diabetes mellitus