Q3 2021 Results

Investor presentation

NOVARTIS | Reimagining Medicine

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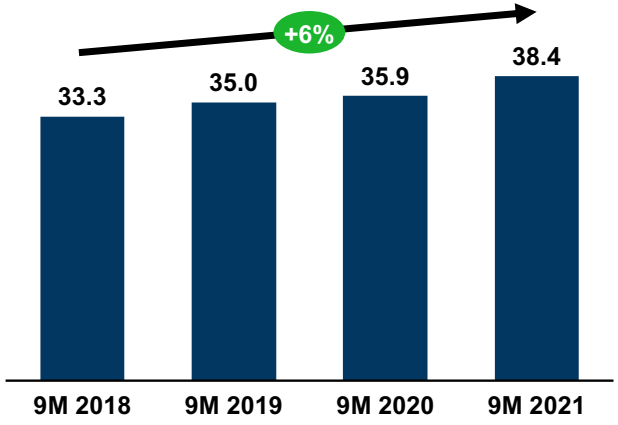
Company overview

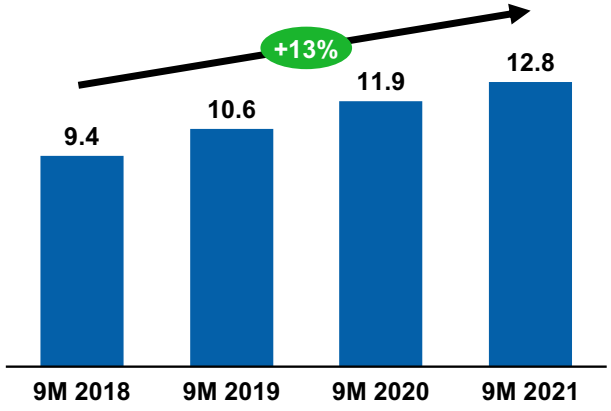
Consistent long-term performance driving confidence for the future

Consistent strong performance since 2018. Confident in our sales growth outlook ~ CAGR 4% to 2025

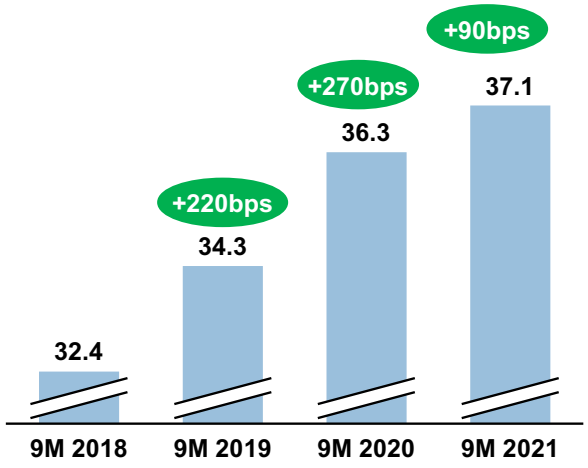
Sales
USD bn, % CAGR cc



Core OpInc
USD bn, % CAGR cc



Innovative Medicines
Core margin (%), growth bps cc



All growth % in cc IM – Innovative Medicines division Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 47 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY

Solid Q3 performance across our value drivers

Growth[1] 1

Q3 Group sales **+5%**; 9M +4%

Q3 IM sales **+7%**; 9M +6%

Q3 Sandoz sales **-2%**; 9M -4%

Innovation 3

^{177}Lu-PSMA-617 mCRPC post-taxane submission (US)

Kymriah® r/r FL submission (US, EU)

Remibrutinib CSU Ph2b showed rapid, effective disease control

Cosentyx® GCA Ph2 TitAIN positive readout

Canakinumab NSCLC 1L, CANOPY1 Ph3 readout

Iptacopan C3G Ph2 final readout (3mo), IgAN addtl analyses (6mo) positive

Productivity[1] 2

Q3 Group core operating income **+9%**; 9M +4%

Q3 IM core operating income **+13%**; 9M +8%

Q3 IM core margin 37.8% (**+1.9%**pts cc); 9M 37.1%

ESG 4

Positive Ph2b for next generation antimalarial therapy

Commitment to **net zero** (2040) with **Science-Based Targets**

Reached **~29m patients** in LMICs to date in 2021 through our flagship program and strategic innovative brands

All growth % in cc IM – Innovative Medicines division BTD – Breakthrough Therapy designation 1. Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 47 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

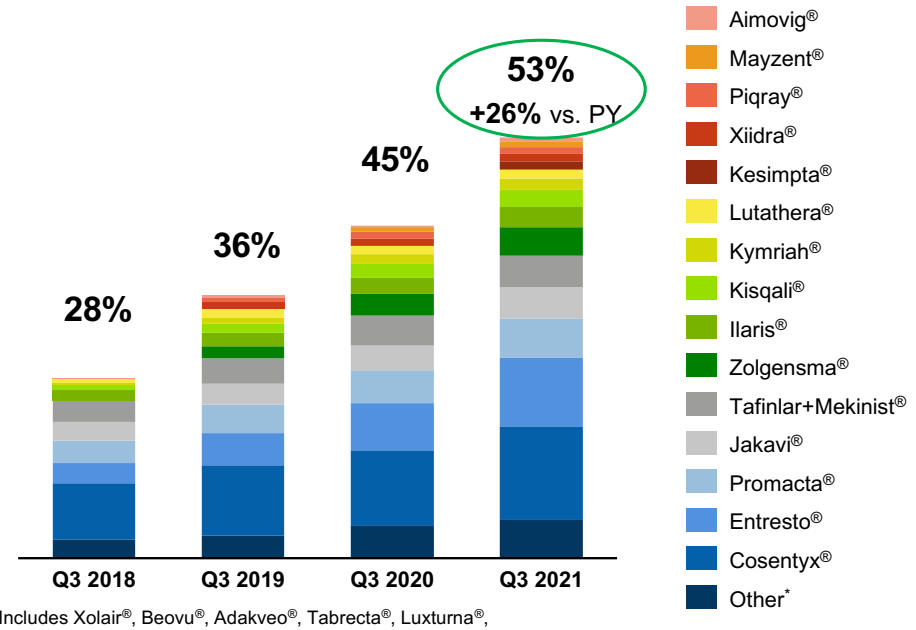
Key growth drivers and launches continue momentum in Q3

Q3 sales[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto® sacubitril/valsartan	924	292	44%
Cosentyx® (secukinumab)	1,247	235	22%
Kesimpta® (ofatumumab)	109	108	nm
JAKAVI® ruxolitinib	426	91	26%
zolgensma®	375	84	28%
PROMACTA® (eltrombopag)	522	80	18%
ILARIS® (canakinumab)	272	52	24%
KISQALI® ribociclib	232	49	27%
Xolair® Omalizumab	365	45	13%
MAYZENT® (siponimod) tablets	76	27	55%
KYMRIAH® (tisagenlecleucel)	146	24	20%
Tafinlar® + Mekinist®	417	20	4%

nm – not meaningful

Key growth drivers and launches 53% of IM sales, growing 26% in Q3



*Includes Xolair®, Beovu®, Adakveo®, Tabrecta®, Luxturna®, Enerzair®, Atectura® and Leqvio®

1. Innovative Medicines division. Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 47 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY

Particularly strong YTD growth for key brands

Cosentyx®	Entresto®	zolgensma®	KISQALI® ribociclib	Kesimpta®
USD 3.5bn	USD 2.6bn	USD 1.0bn	USD 0.7bn	USD 0.2bn
+18%	+41%	+49%	+27%	nm

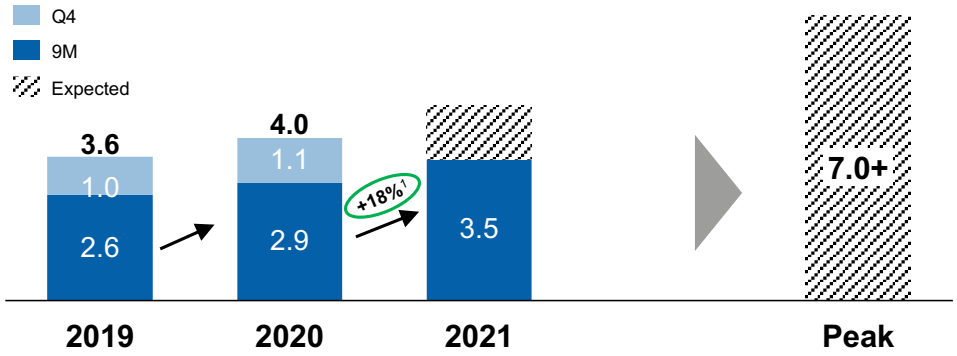
All growth rates in constant currencies (cc)

Raising peak sales guidance for Cosentyx® to at least USD 7bn and Entresto® to at least USD 5bn

Cosentyx®

Expected peak sales **USD ≥ 7bn**

Sales evolution (USD bn)
Illustrative



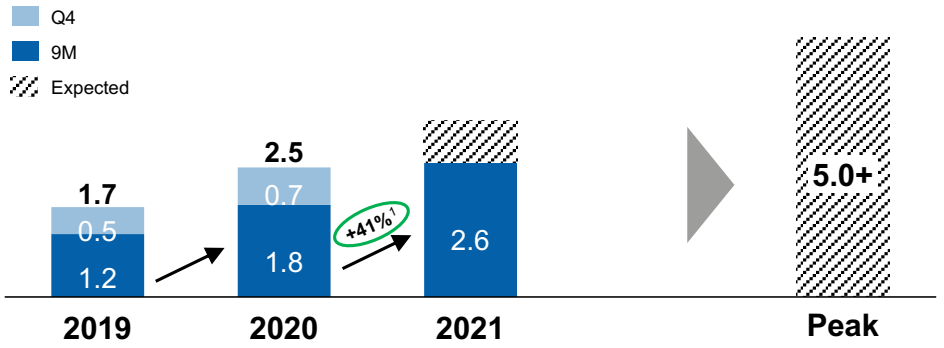
Market growth, geographic expansion, LCM

- Market growth driven by increasing use of biologics
- Geographic expansion continues in China
- Expansion to 10+ potential indications including HS, LP, LN, GCA
- 3+ potential formulations and dosing enhancements including i.v.

Entresto®

Expected peak sales **USD ≥ 5bn**

Sales evolution (USD bn)
Illustrative



Market penetration, guidelines, geographic expansion

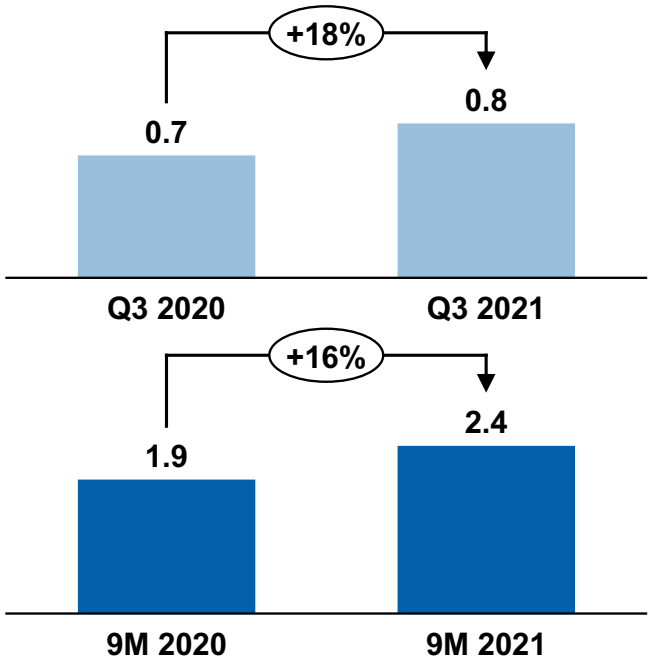
- Only 30% of HFrEF patients receive Entresto®
- Expanded label increases eligible US patient population to 5m patients
- Guidelines positioning Entresto® as first line therapy
- Significant opportunity in China and Japan, both including hypertension

1. 9M vs PY

China sales on track to double by 2024

Group sales evolution

USD bn, % cc



Strong performance of growth drivers following NRDL inclusion[1]

- Solid momentum for Cosentyx® and Entresto®
- All key products included in NRDL, outperforming multinational companies by sales growth for those brands
- Limited exposure to VBP

Expanding commercial footprint for additional reach

- Ramping up activities in lower tier cities and hospitals
- Broadening presence in retail and e-pharmacy channels
- Increasing cross-functional account management for higher productivity

Late stage pipeline to fuel further growth

- Entresto® HTN, Lucentis® PDR/ROP, Cosentyx® PedPsO approved in Q3
- Tafinlar®+Mekinist® lung cancer, Kisqali® submitted in Q3
- Additional ~50 submissions planned in next 5 years

NRDL – National Reimbursement Drug List IL – Interleukin HTN – Hypertension PDR/ROP – Proliferative Diabetic Retinopathy/ Retinopathy of Prematurity PedPsO – Pediatric Psoriasis 1: Entresto® NRDL inclusion in Q1/2020, Cosentyx® in Q1/2021

Sandoz performance ex-US normalizing, growing +3% in Q3

Q3: Biosimilars, Retail in Europe and ROW gaining share

Q3 sales USD 2.4bn (-2% cc)

- **Europe +2%** (56% of sales)
  Bio, Retail, gaining market share
- **ROW +6%** (26% of sales)
  Steady growth across regions, Biosimilars and Retail
- **US -20%** (18% of sales)
  Price erosion, contract terminations

Q3 Core OpInc 0.6bn (-15%)

- Unfavorable gross margin in US

Q4: Performance normalizing ex-US as COVID-19 impact decreases

Direct demand

- Different business segments normalizing at **different rates**
- Demand impact in Q4 expected to be **in line with Q3**

Cough & Cold

- Could start to **return to pre-Covid levels** in several markets

Losartan

- Negative impact of recall

All growth rates in constant currencies unless otherwise stated STO – Sandoz Technical Operations

Commencing strategic review of Sandoz

Gx market attractive, Sandoz well placed to capitalize on growth drivers over next decade. Preparing for accretive growth with next wave of Biosimilar launches.

Attractive market

- Gx business **attractive**: **>USD 400 bn sales** LOE over next 10 years (>**170 bn in biologics**)
- **Gx market estimated CAGR** ~4% to 2026 (**biosimilar CAGR** ~9%)
- Expect gradual **post-COVID rebound**, particularly antibiotics

Strong presence

- **#1 in Europe,** only Gx MNC with top 5 position in all main regions
- **Europe** growing, **Emerging Markets** accelerating, focus to stabilize US
- **Global leadership** positions in biosimilars (**8 in market**), Gx antibiotics, Gx oncology

Strategic focus

- **Biosimilars pipeline doubled**[1]; **15+ assets** in development. Aiming for USD 3bn sales by 2025, USD 5bn by 2030
- **Complex small molecules:** e.g. antibiotics, oncology, respiratory, injectables
- **Ongoing COGS improvements:** autonomous Sandoz Technical Operations
- **Aspiration** to become global Gx #1, building on brand, reach, scientific and commercial expertise

Strategic review to maximize shareholder value: Review will explore all options, ranging from retaining business to separation. Update on progress of the review to be provided latest at the end of 2022.

1. Over last 3 years

Broad pipeline of novel medicines continued to progress in Q3

Approvals

Cosentyx®	JP, CN: pediatric PsO
Entresto®	JP: essential hypertension

Readouts and publications (selected)

●	**Remibrutinib**	Ph2 – CSU
●	**Canakinumab**	Ph3 – NSCLC 1L[1]
●	**Kisqali®**	Ph3 – aBC OS results (MONALEESA-2)
●	**Cosentyx®**	Ph2 – GCA (TitAIN)
●	**Iptacopan**	Ph2 – C3G
●	**Iscalimab**	Ph2 – kidney Tx
●	**Kymriah®**	Ph2 – aNHL 2L (BELINDA)
●	**BYL719**	Real world – PROS (EPIK-P1)
●	**Ganaplacide**	Ph2b – Malaria

● Negative ● Positive

Submissions

^{177}Lu-PSMA-617	US: mCRPC, post-taxane
Beovu®	US, EU, JP: diabetic macular edema
Tislelizumab	US: 2L ESCC
Kymriah®	US, EU: r/r follicular lymphoma
Asciminib	JP: chronic myeloid leukemia, 3L
Tafinlar®	US: tumor agnostic BRAF mutation

Designations

Sabatolimab	EU orphan drug designation, MDS
Asciminib	FDA priority review, CML 3L
^{177}Lu-PSMA-617	FDA priority review, mCRPC, post-taxane
LNA043	FDA fast track designation, knee osteoarthritis
NIS793	FDA orphan drug designation, pancreatic cancer 1L

See last slide for all abbreviations 1. Post Q3 event. Ph3 study did not meet primary endpoints. PFS and OS trends support further evaluation with additional analyses ongoing

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

Selected assets

Cardio-renal

Leqvio®	Hyperlipidemia: FDA action date January 1, 2022
	CVRR-LDLC: Ph3 ongoing
Iptacopan	IgAN, C3G, aHUS: Ph3s started 2021
	iMN: Ph2b ongoing
Pelacarsen	CVRR-Lp(a): Ph3 ongoing

Immunology

Cosentyx®	HS: Ph3 SUNRISE, SUNSHINE readout H2 2021
	GCA: Ph2 readout positive
	jPsA/ERA (submitted), lupus nephritis (Ph3), lichen planus (Ph2)
Ligelizumab	CSU: Ph3 PEARL 1, 2 readout 2021[1]
	CINDU, food allergy[2]: Ph3 starts H2 2021
Remibrutinib	CSU: Ph2 data positive
	Other indications being explored
Ianalumab	Sjögren's, SLE, Autoimmune hepatitis: Ph2s ongoing
Iscalimab	Sjögren's, liver Tx, HS: Ph2s ongoing

Neuroscience

Zolgensma®	SMA IT: FDA hold lifted, Ph3 initiating
Branaplam	Huntington's disease: Ph2b start H2 2021
Remibrutinib	Multiple sclerosis: Ph3 start H2 2021

'Wild Cards'

LNA043 (osteoarthritis: Ph2 ongoing), CSJ117 (asthma: Ph2 ongoing, COPD Ph2 started), QBW251 (COPD: Ph2 recruitment completed), SAF312 (COSP: Ph2 SAHARA ongoing), UNR844 (presbyopia: Ph2 READER ongoing)

1. Q4/2021-Q1/2022 potential COVID-19 impact 2. Food Allergy indication falls within the Respiratory & Allergy therapeutic area

… and strength and depth in oncology

Selected assets

Solid tumors

Kisqali®	HR+/HER2- BC (adj) NATALEE readout event-driven, expected end 2022
Canakinumab	NSCLC adjuvant: Ph3 ongoing
^{177}Lu-PSMA-617	mCRPC post-taxane: submitted
	mCRPC pre-taxane: Ph3 started
	mHSPC: Ph3 started
JDQ443 KRAS inhibitor	2/3L NSCLC: Ph3 start in H1 2022
TNO155 SHP2 inhibitor **combinations**	Solid tumors: multiple combinations being explored in ongoing trials
Tislelizumab	2L esophageal cancer: submitted
	NSCLC: H1 2022 MAA submission, evaluation of US BLA submission options ongoing. Multiple indications in Ph3

Hematology

Asciminib	CML 3L: submitted
	CML 1L: Ph3 started
Iptacopan	PNH: Ph3 started
Sabatolimab	HR-MDS: Ph2 STIMULUS-MDS-1 continues to PFS readout[1]
	Ph3 STIMULUS-MDS-2 ongoing
	AML: Ph2 STIMULUS-AML-1 ongoing
YTB323 CD19 CAR-T	r/r DLBCL: Ph2 (pivotal) start 2022

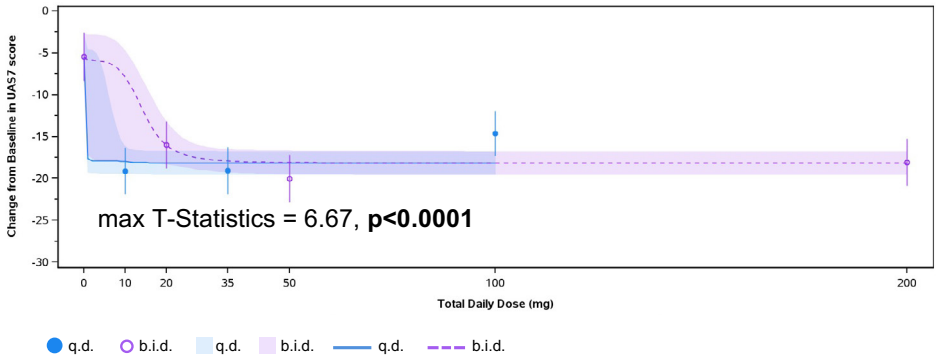
'Wild Cards'

LXH254 (melanoma: combo Ph2, NSCLC combo Ph1), NIS793 (mPDAC: Ph3 started, colorectal cancer Ph2 initiating)

1. 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

Remibrutinib maintained robust clinical efficacy throughout treatment period, with fast onset of action in CSU (1/3)

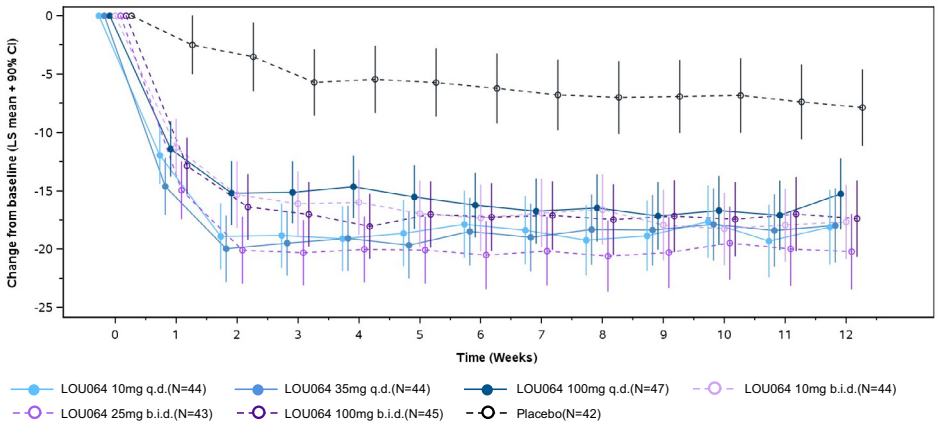
Dose-response with significant improvements vs. placebo



Primary endpoint met; dose-response vs. placebo UAS7 change from baseline at Week 4

No safety signals

Rapid and significant improvement in UAS7 over 12 weeks vs. placebo



UAS7 **scores improved from baseline up to Week 12** for all doses compared with placebo

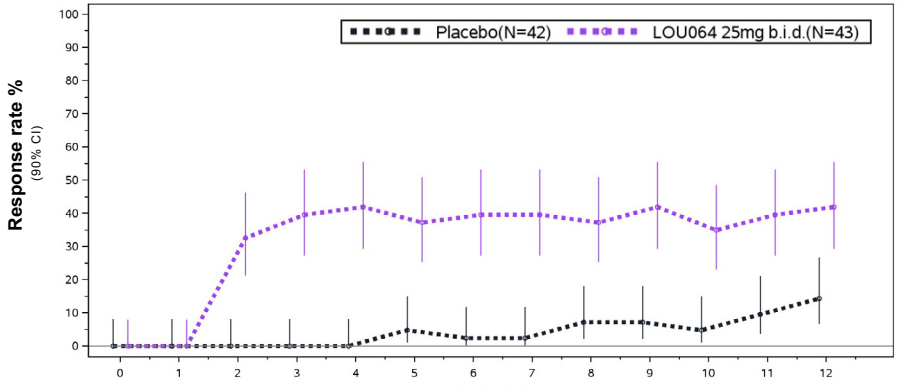
Improvement was rapid (UAS7) as **early as Week 1**, and **maintained up to Week 12**

AE – Adverse events CSU – chronic spontaneous urticaria UAS7 – weekly Urticaria Activity Score b.i.d. – two times a day

Favorable benefit/risk profile across the entire dose range, with no dose-dependent pattern of AEs (2/3)

More patients achieved complete control (UAS7=0)

UAS7=0 vs. placebo (n=43)



- **More patients** on remibrutinib achieved complete control, i.e. **complete absence of hives and itch** (UAS7=0) over 12 week treatment period
- **High response rate** maintained, up to end of treatment

Remibrutinib demonstrated good tolerability across the entire dose range tested with no safety signals

Key safety data include:

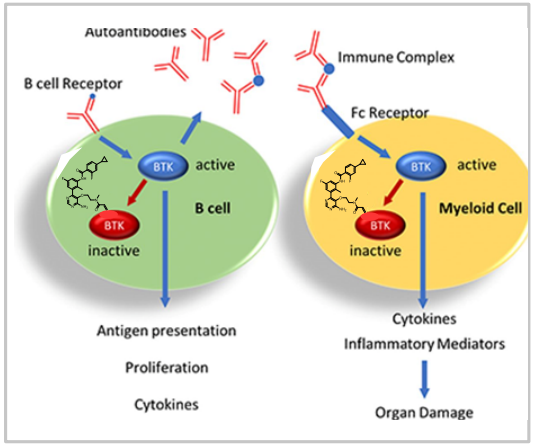
- ✓ No dose dependent increase of, treatment interruption or discontinuation due to LFT elevations
- ✓ No dose dependent cytopenias, treatment interruption or discontinuation due to low blood cell counts
- ✓ No clinically relevant adverse events associated with BTK inhibitor class (e.g. infections, cytopenias, bleeding, hepatic events) across the dose range tested

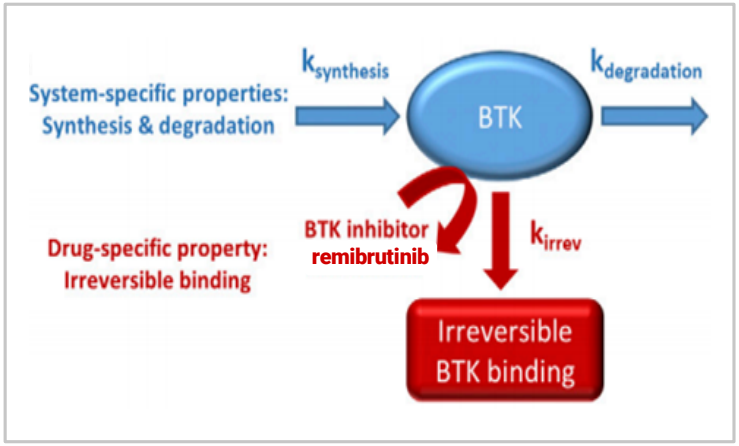
First oral therapy to advance to Ph3 in CSU in 2021 in H1 antihistamines inadequate responders.
Best-in-class profile based on positive benefit/risk profile. Ph3 studies in CSU expected to start Q4 2021

AE – Adverse events CSU – chronic spontaneous urticaria UAS7 – weekly Urticaria Activity Score b.i.d. – two times a day

Initiating Ph3 trials with remibrutinib in relapsing multiple sclerosis (3/3)

Remibrutinib, potential best-in-class potency, selectivity and safety. May offer a **more comprehensive and sustained BTK inhibition** that allows for **maximizing efficacy** without compromising patient safety





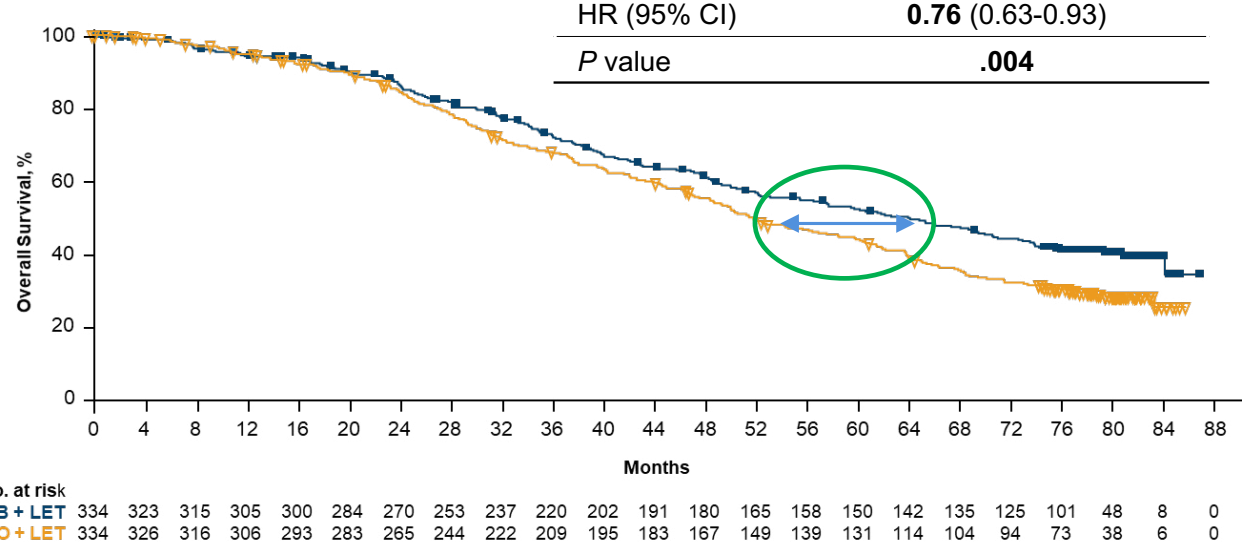
- **Covalently binds** to the intracellular enzyme BTK in B cells and myeloid cells
- **Potent BTK inhibition** with brief and low systemic exposure which **minimizes risk** for AEs and drug-drug interactions
- CSU data – **no dose-limiting side effects** in Ph2a trial
- **Move directly into Ph3** in MS, with trials to start in Q4 2021
- Novartis MS portfolio offers treatment options across the MS spectrum

Kisqali® achieved statistically significant OS benefit in MONALEESA-2

Improvement in median OS was **12.5 months** with Kisqali ® plus letrozole

Presented at ESMO 2021

	RIB + LET	PBO + LET
Events/n	181/334	219/334
Median OS, mo	**63.9**	51.4
HR (95% CI)	**0.76** (0.63-0.93)	
P value	**.004**	



- **Longest median OS in advanced breast cancer (>5 yrs)**
- Significant survival benefit **(>1 yr)**
- + endocrine therapy the **only first-line treatment with OS benefit**
- Considered as **preferred treatment option** for HR+/HER2− aBC
- Planning to submit this OS data to be incorporated into drug labels

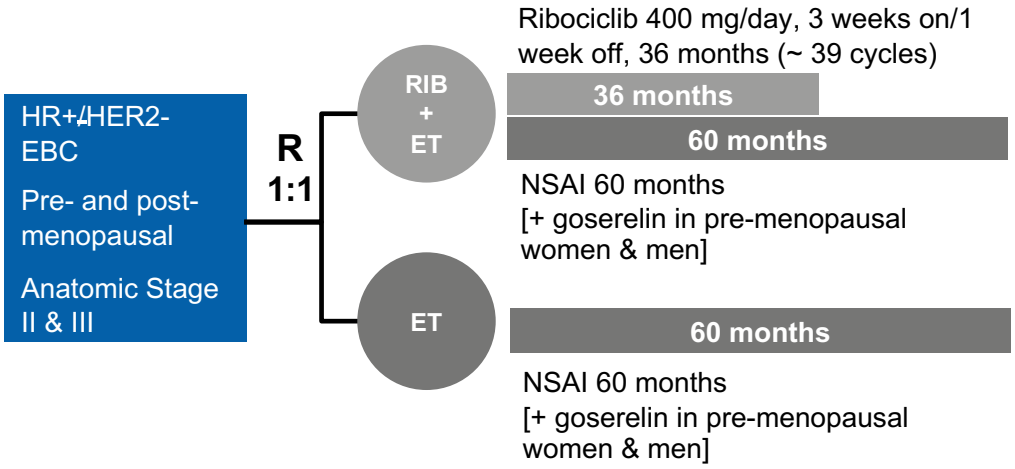
aBC – advanced breast cancer

Kisqali® is being investigated in HR+/HER2- early breast cancer (adjuvant) in the Ph3 NATALEE study, expected readout in 2022

NATALEE study design

Primary endpoint: invasive disease-free survival (iDFS), event driven

Sample size increased to 5000 – more robust



Early Breast Cancer (EBC)

- 83% of breast cancers are diagnosed as EBC
- EBC treatment objective: cure the patient by preventing disease recurrence while maintaining QoL

What makes NATALEE unique?

- Includes patients with high and intermediate risk of recurrence based on AJCC prognostic staging
- Longer treatment duration: 3 vs. 2 years
- Lower dose compared to metastatic setting (400 vs. 600mg) may improve overall tolerability

Study status

- Enrollment complete; readout event-driven and expected late 2022; planned submission 2023
- FDA feedback confirms iDFS acceptable as primary analysis provided no detriment in OS

CANOPY-1 Ph3 data support further evaluation of canakinumab in lung cancer

CANOPY-1

- Did not meet primary endpoints: OS and PFS in previously untreated locally advanced or metastatic NSCLC[1]
- **Potentially clinically meaningful improvements in both PFS and OS** among pre-specified subgroups of patients with inflammatory biomarkers; additional analyses ongoing[1]
- Results support **continued study of canakinumab in earlier stages** of lung cancer, further evaluation of Pro-Tumor Inflammation in all lung cancer settings[1].
- **CANOPY-A** study more closely reflect the CANTOS study population vs. CANOPY-1[2,3,4]. CANTOS the 1st study to suggest that IL-1β inhibition may play a role in lung cancer[3]
- **No unexpected safety signals** when combined with pembrolizumab plus platinum-based chemotherapy

Study	Patient population	Hypothesis	Findings
CANOPY-2 2/3 L NSCLC	Metastatic NSCLC treatment failed. Canakimumab with docetaxel	Reduce progression of treatment resistant tumors	Primary endpoint OS not met. No benefit observed.
CANOPY-1 1L NSCLC	Metastatic NSCLC, treatment naive. Canakimumab combined with pembrolizumab	Reduce progression of treatment naive tumors	Primary endpoints of OS + PFS not statistically significant. Potentially clinically meaningful OS +PFS improvements in pre-specified subgroups (hs-CRP, other biomarker-defined subgroups)
CANOPY-A Adjuvant NSCLC	Stage II-III NSCLC. Canakimumab after complete resection and adjuvant chemotherapy	Reduce development of tumors from micro-metastatic disease.	To be determined
CANTOS	Stable post MI with elevated hsCRP > 2mg/L	Alter tumor development in high risk population	Dose-dependent reduction in fatal/non-fatal lung cancer incidence

Developing other potential pro-tumor inflammation pathway inhibitors, which are at various stages of development, incl. gevokizumab[5,6]

See appendix for references NSCLC – Non-small cell lung cancer OS – Overall survival PFS – Progression-free survival

Accelerated ESG efforts toward increasing access to medicines, improving health equity and achieving net-zero carbon emissions

1
Access in LMICs

Reached nearly **29m patients** to date in 2021 through our flagship programs and strategic innovative brands

2
R&D

Achieved positive Ph2b for **next generation antimalarial therapy** ganaplacide in combination with lumefantrine

3
Health equity

Announced a planned **10-year commitment** with historically black colleges and universities in US to address **root causes** of systemic disparities in health outcomes

4
Environment

Committed to achieving **net zero** carbon emissions based on **Science-Based Targets** across our value chain by **2040**

Marie-France Tschudin

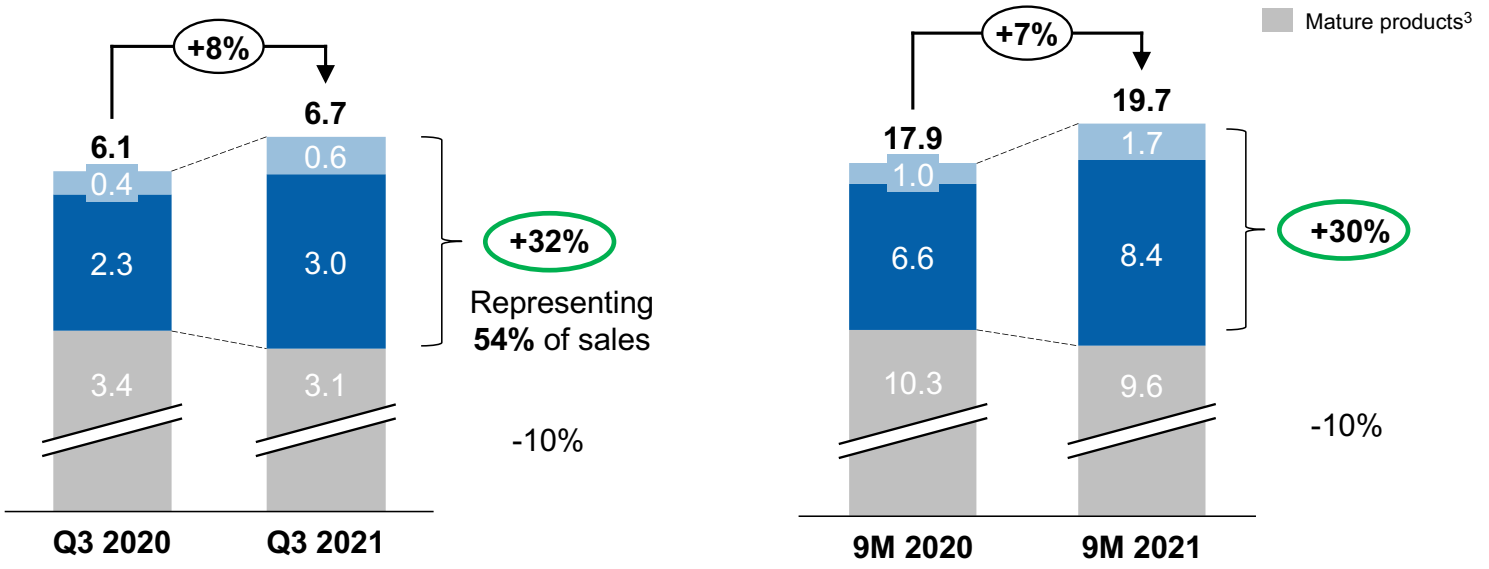
President, Novartis Pharmaceuticals

Pharmaceuticals grew +8% in Q3 with growth drivers and launches showing strong momentum

Growth drivers and launches +32% in Q3, representing 54% of sales (up from 44% Q3 2020)

Pharmaceuticals net sales

USD bn, % cc

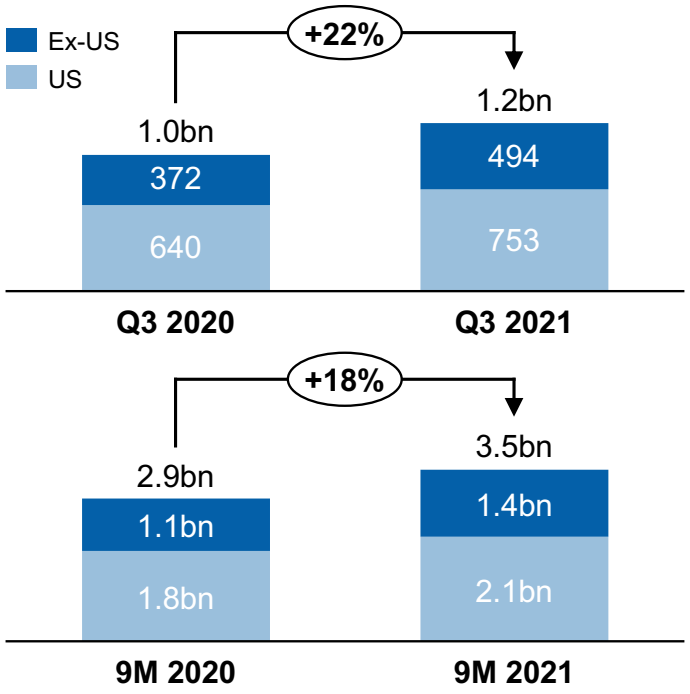


All % growth relate to cc unless otherwise stated 1. Zolgensma®, Kesimpta®, Mayzent®, Beovu® , Luxturna®, Leqvio®, Enerzair® and Atectura® 2. Cosentyx®, Entresto®, Xolair® , Ilaris® , Xiidra® and Aimovig® 3. All other brands.

Cosentyx® grew 22% in Q3; label expansion with pediatric approvals

Sales evolution

USD m, % cc



Strong growth across core indications and markets

- US: growing volume in line with market across indications vs. Q2
- EU: leading biologic in PsO, leading originator biologic in SpA[1]

Expanding clinical differentiation with pediatric approvals

- Ped PsO approval in China
- 75mg PFS approval in EU for patients <50kg
- JPsA and ERA indications granted priority review by FDA

Ped PsO – Pediatric Psoriasis PsO – Psoriasis SpA - Spondyloarthritis PFS – Pre-filled Syringe jPsA – Juvenile psoriasis arthritis ERA – Enthesitis related rheumatoid arthritis 1. EU patient share data June 2021.

Cosentyx® peak sales expectations of at least USD 7bn driven by market growth, geographic expansion and LCM

In-market indications

USD 29bn market growing double-digit[1]

Current biologics treated: 15% PsO, 13% axSpA, 23% PsA[2]

China

China post-NRDL acceleration with potential to expand to PsA and nr-axSpA

Lifecycle management

Expansion to 10+ potential indications:

Hidradenitis Suppurativa (Ph3 readout Q4), GCA (Ph2 positive, Ph3 started), Lichen Planus (Ph2), Lupus Nephritis (Ph2)

Additional potential label enhancements including i.v. for PsA (Ph3 positive) & AS, 300mg autoinjector, PsO flex dosing

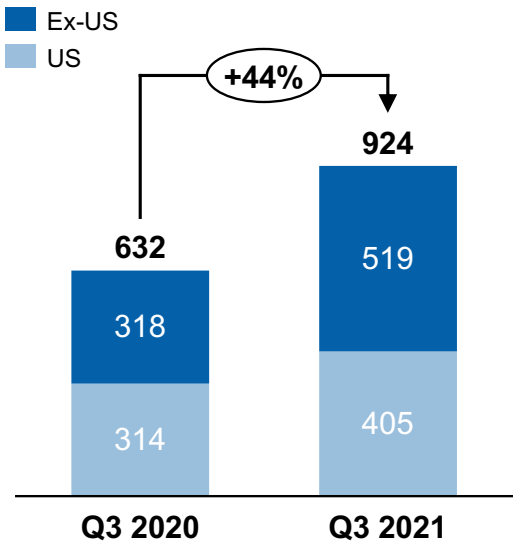
People + excellence in operations

See appendix for references PsO – Psoriasis axSpA – axial Spondyloarthritis nr-axSpA – non-radiographic axial Spondyloarthritis PsA – Psoriatic Arthritis NRDL – National Reimbursement Drug List AS – Ankylosing Spondylitis

Entresto® grew +44% in the quarter driven by expanded label in US, guideline[1,2] uptake and strong execution

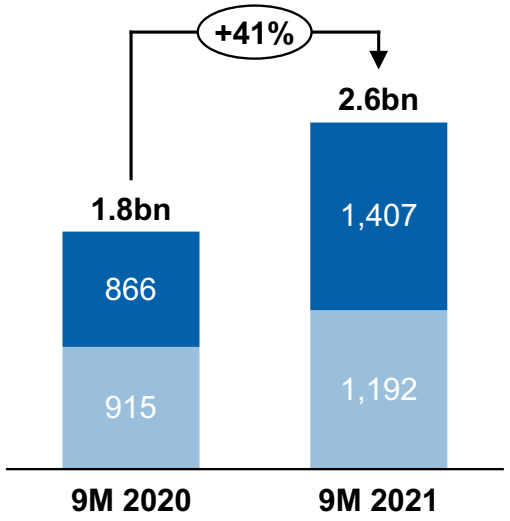
Sales evolution

USD m, % cc



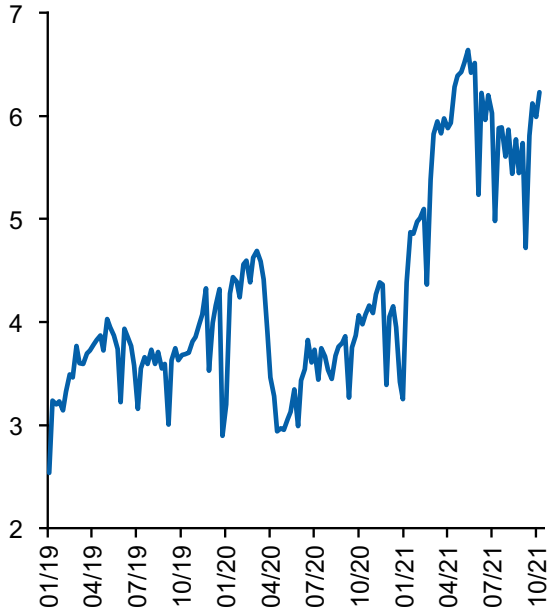
YTD sales growth vs. PY

US +30%, ex-US +53%



US weekly NBRx[3]

New-to-brand prescriptions (000)



See appendix for references

Entresto® peak sales expectations of at least USD 5bn driven by remaining patient potential, guidelines, geographic expansion

In-market indications

~70% of eligible HFrEF patients not currently treated in G7[1]

US expanded label increased addressable population to 5m

Guideline implementation

ACC ECDP, ESC and national HF guidelines[2] recommend ARNI 1L

Entresto to replace ACE/ARB in all appropriate HFrEF patients

Partnerships to implement quality metrics for HF care

Geographic expansion

Strong momentum in HFrEF in China

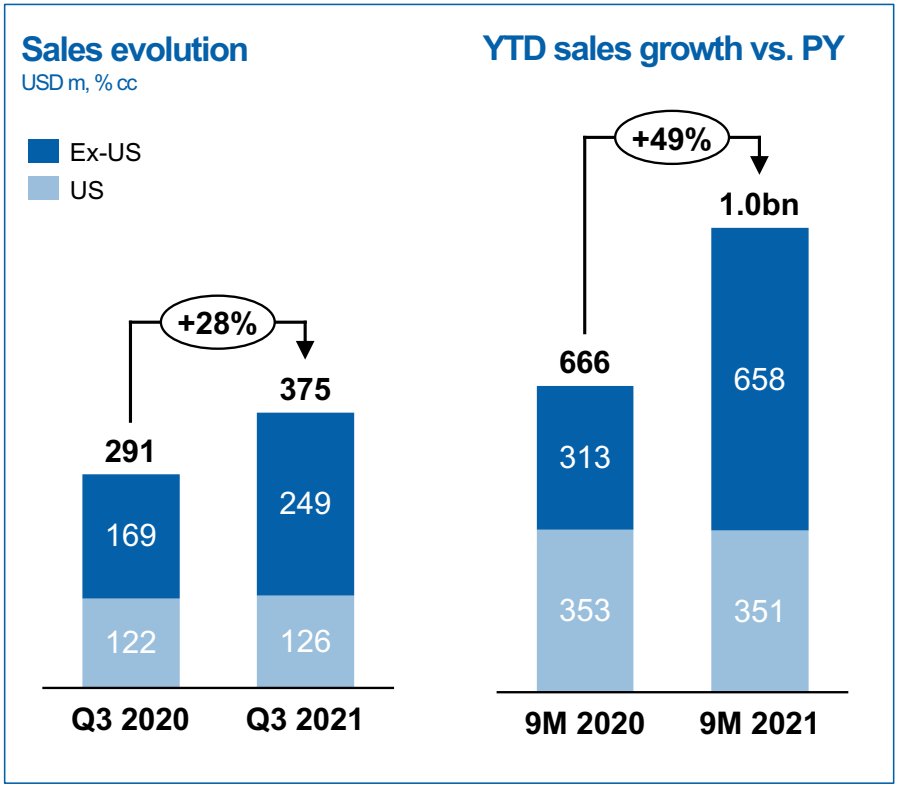
Long-term growth in Japan across CHF and HTN

Additional growth outside of G7 based on expanded HF label and HTN

People + excellence in operations

See appendix for references HFrEF – Heart Failure with reduced Ejection Fraction ACC - American College of Cardiology ECDP – Expert Consensus Decision Pathway ESC – European Society of Cardiology HF – Heart Failure ARNI – Angiotensin Receptor Neprylisin Inhibitor ACE – Angiotensin Converting Enzyme ARB – Angiotensin II Receptor Blocker

Zolgensma® grew 28% in the quarter mainly due to geographic expansion, YTD sales over a billion



Q3 highlights

- Growth driven by expanding access in EU, emerging markets
- Steady US sales continue to be driven by incident patients
- Access pathways in 23 countries
- 1.6k+ patients have been treated with Zolgensma® worldwide[1]

2021 growth drivers

- Reimbursement: Implementation of recent agreements (i.e. Russia, BeneluxA)
- Newborn screening: ~84% in US; on track for 20% in EU end 2021

Advancing robust data in SMA

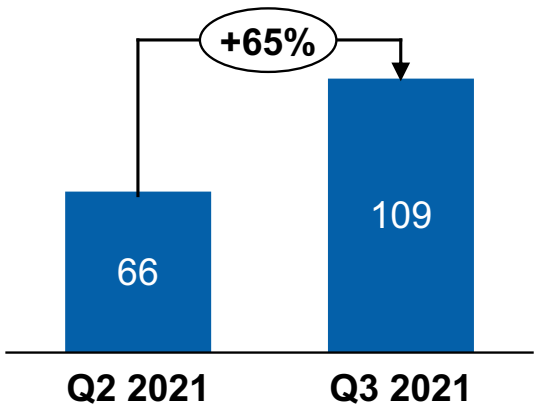
- **STEER** (global Ph3 OAV101 IT in patients ages 2-18 with later-onset SMA): anticipate screening first patients by end 2021
- **SMART** (Ph3b study for Zolgensma IV in children up to 21 kg[2]): rapid enrollment reflects real-world interest and use

BeneluxA – Belgium, Netherlands, Luxembourg, Austria 1. Commercially, via managed access programs and in clinical trials 2. SMART is enrolling children ≥ 8.5 kg and ≤ 21 kg

Kesimpta® accelerating launch momentum driving B-cell market growth

Sales evolution

USD m, % cc



Solid launch execution in US

- **Strong contributor to B-cell** therapy market growth
- **NBRx share 12.7%[1], 2nd** highest ahead of Aubagio® and Tecfidera®
- **>6k** patients treated, **majority** naive or first switch
- **+34% prescribers** in the quarter

Continuing clinical differentiation

- **ALITHIOS: IgG levels preserved** over 3.5 years with low risk of serious infections
- 94% COVID-19 cases mild / moderate in unvaccinated treated adults
- **Ongoing / initiating studies** include immune response to SARS-CoV-2 mRNA vaccines, efficacy and safety of switching from other therapies, investigating PROs in early RMS population in real-world setting

IgG – Immunoglobulin G PIRA – Progression independent of relapse activity PROs – Patient reported outcomes 1. Unadjusted exit share by end of Q3

Leqvio® on track for US launch with FDA action date January 1; As with other cardiovascular launches, expect slow initial ramp

Launch preparation

Enable health system readiness

- Ensure protocols in place to identify and manage patients in ~200 prioritized systems

Drive awareness among HCPs

- Education: unmet need, importance of LDL-C
- Leveraging strong CV footprint

Facilitate product acquisition

- Field team helping to navigate access complexities
- Network of >1000 AICs to support customers new to buy-and-bill

Evidence generation

V-INITIATE

Explore "Leqvio® first" strategy directly after statins[1]

V-INCEPTION

Investigate Leqvio® initiation after recent ACS events[1]

H1 2022 expectations

- High interest from early adopters
- Independent HCPs ready for buy-and-bill
- AICs – responding to demand
- Temporary J-code

H2 2022 expectations

- Permanent J-code available
- Buy-and-bill capabilities established
- System P&T committee review complete
- Finalization of payer coverage policies

See appendix for references HCP – Healthcare Professional ASCVD – Atherosclerotic Cardiovascular Disease LDL-C – Low Density Lipoprotein Cholesterol AIC – Alternative Injection Center P&T – Pharmacy & Therapeutics Committee

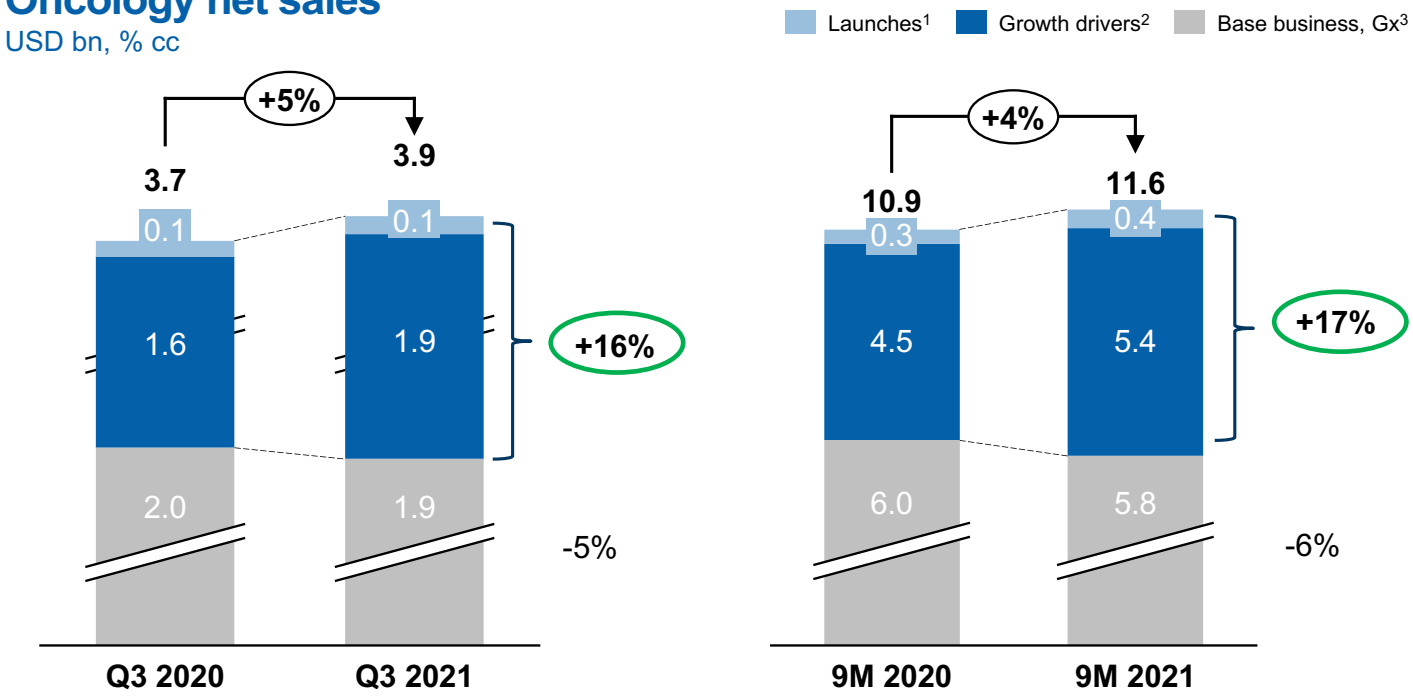
Susanne Schaffert

President, Novartis Oncology

Oncology grew 5% in Q3 driven by strong execution

Oncology net sales

USD bn, % cc



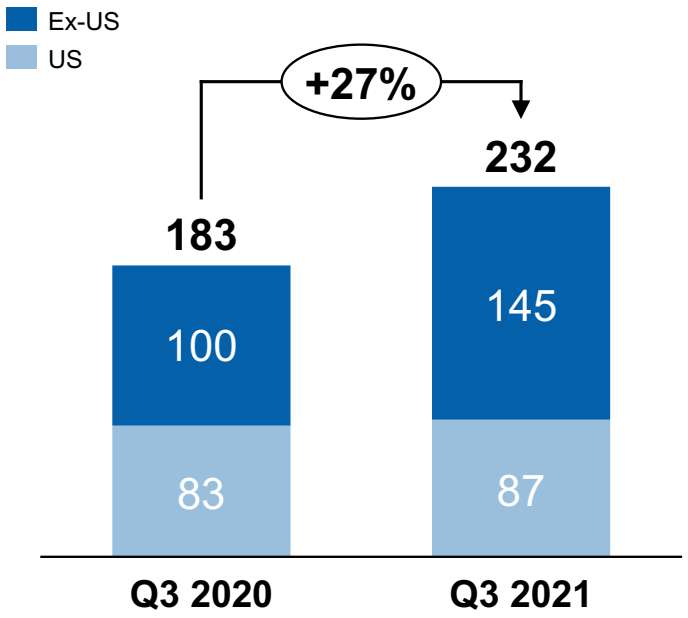
- Growth drivers and recent launches contribute **52%** of sales (vs. 47% in Q3 2020)
- Strong double-digit growth for Jakavi®, Promacta®/ Revolade® and Kisqali®
- COVID-19 situation normalizing with patient visits, diagnosis and treatment rates increasing, but still below pre-COVID

1. Launches include Piqray®, Adakveo® and Tabrecta® 2. Growth drivers include Promacta®/Revolade®, Tafinlar®+ Mekinist®, Kisqali®, Lutathera®, Kymriah® and Jakavi® (marketed by Novartis ex-US). 3. Base business – other brands. Gx include Afinitor®, Exjade® / Jadenu®, Glivec® and Sandostatin® All % growth relate to cc unless otherwise stated.

Kisqali® grew +27% in Q3; MONALEESA-2 OS results reinforcing best-in-class profile

Sales evolution

USD bn, % cc



Kisqali® well positioned to become first-choice CDK4/6i

- Only CDK4/6i with positive OS results in 3 Ph3 trials
- MONALEESA-2 unprecedented OS results in largest segment of aBC population

Continued growth acceleration, with share gains ex-US

- Ex-US: Market leader in pre-menopausal setting in EU4 and UK
- US demand in pre-menopausal and post-menopausal picking up

Moving forward with confidence in Kisqali® and its unique profile

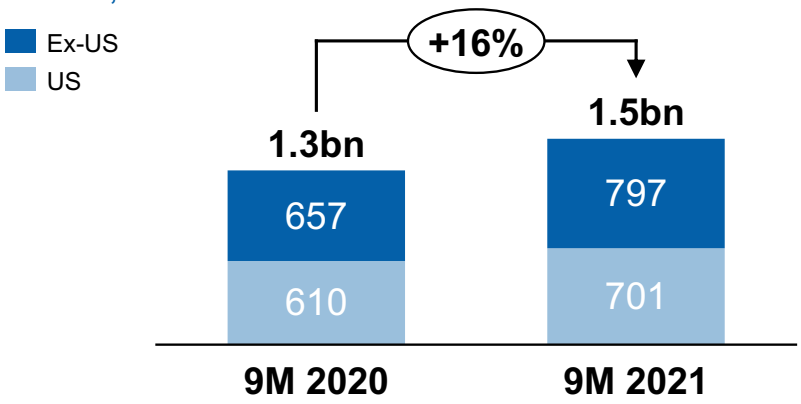
- HARMONIA study to evaluate ribociclib vs. palbociclib in aggressive HER2-enriched intrinsic subtype of HR+/HER2- aBC
- NATALEE study in eBC completed enrollment in Q2 2021; readout event-driven and expected end 2022

aBC – advanced breast cancer eBC – early breast cancer

Continued double-digit growth with Promacta®/Revolade® and Jakavi® reflect strong execution

Promacta®/Revolade®

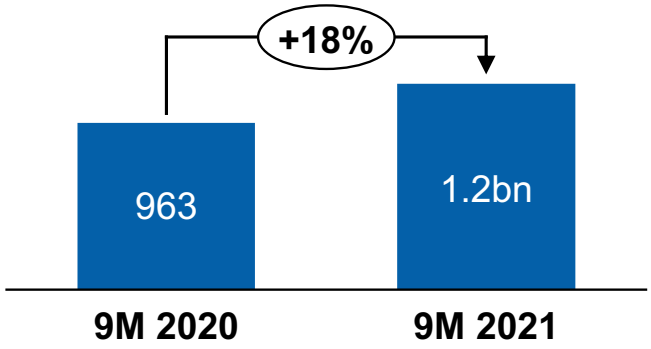
USD m, % cc



- Most used thrombopoietin receptor agonist with the broadest indication
- +10% US NBRx share increase[1] driven by sustained efficacy, oral convenience and non-immunosuppressive profile
- US indication expansion to include persistent ITP expected to fuel further growth

Jakavi®

USD m, % cc



- Standard-of-care JAK inhibitor in MPN with proven overall survival and quality of life benefit
- Strong double-digit growth across regions driven by earlier usage in myelofibrosis and polycythemia vera
- Preparing for launch of acute and chronic GvHD in 2022

MPN – Myeloproliferative neoplasms ITP – Immune thrombocytopenia SAA – Severe aplastic anemia GvHD – Graft versus host disease 1. Data: IQVIA data, August Rolling 3-month data vs last year August

Preparing to launch our next growth drivers Scemblix® and ^{177}Lu-PSMA-617 in H1 2022

Asciminib
Scemblix®[1]

- First STAMP inhibitor, with potential to transform standard of care in CML
- Well positioned to launch in 3L CML with 80% pre-launch awareness
- FDA and EMA 3L CML submission in June 2021
- With FDA BTD and Fast Track designation, US approval expected by end of Q1 2022
- 1L pivotal study initiated

^{177}Lu-PSMA-617

- VISION trial showed significant OS and rPFS benefit in advanced mCRPC
- Awareness campaign on PSMA & Phenotypic Precision Medicine
- Educating community centers on requirements for RLT
- With FDA Priority Review, PDUFA expected H1 2022; EMA submission on track
- Submitted ^{68}Ga-PSMA-11 kit for PET imaging to FDA, EMA submission on track
- Moving into earlier lines with Ph3 studies in mHSPC and mCRPC pre-taxane initiated

1. The brand name Scemblix® has been provisionally approved by the FDA for the investigational product asciminib (ABL001), but the product itself has not been approved for sale in any country PSMA – Prostate-specific membrane antigen
FPFV – First patient first visit RLT – Radioligand therapy rPFS – Radiographic progression-free survival STAMP – Specifically targeting the ABL myristoyl pocket

Harry Kirsch
Chief Financial Officer

Financial review and 2021 guidance

Delivering solid results in the quarter; sales +5%, core OpInc +9%

Group[1] USD million	Q3 2021	Change vs. PY		9M 2021	Change vs. PY	
		% USD	% cc		% USD	% cc
Net Sales	13,030	6	5	38,397	7	4
Core Operating income	4,467	10	9	12,769	7	4
Operating income	3,233	34	32	9,127	22	18
Net Income	2,758	43	41	7,712	29	26
Core EPS (USD)	1.71	13	11	4.88	10	7
EPS (USD)	1.23	45	44	3.44	31	28
Free Cash Flow	4,423	64		10,255	23	

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 47 of the Condensed Interim Financial Report. All % growth relate to cc unless otherwise stated.

Driven by strong performance of Innovative Medicines, with double digit core OpInc growth in the quarter

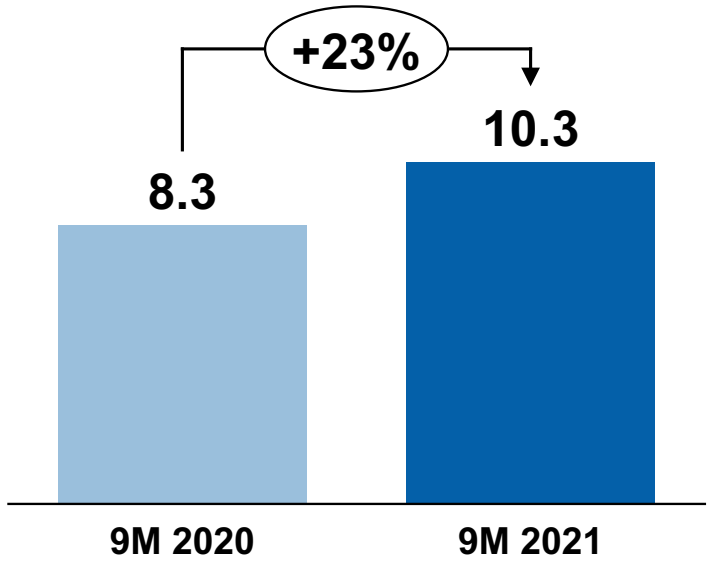
	Q3 2021				9M 2021			
	Net sales change vs. PY	Core operating income[1] change vs. PY	Core margin[1]	Core margin[1] change vs. PY	Net sales change vs. PY	Core operating income[1] change vs. PY	Core margin[1]	Core margin[1] change vs. PY
	(in % cc)	(in % cc)	(%)	(%pts cc)	(in % cc)	(in % cc)	(%)	(%pts cc)
Innovative Medicines	7	13	37.8	1.9	6	8	37.1	0.9
Sandoz	-2	-15	23.8	-3.6	-4	-18	21.6	-3.7
Group	5	9	34.3	1.0	4	4	33.3	0.1

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 47 of the Condensed Interim Financial Report.

9M 2021 free cash flow growing to USD 10.3bn

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

\+ Favorable working capital

\+ Higher divestment proceeds

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

1. Free cash flow is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 47 of the Condensed Interim Financial Report.

2021 Novartis full year guidance

Barring unforeseen events; growth vs. PY in cc

Group	**Sales expected to grow low to mid single digit** **Core OpInc expected to grow mid single digit, ahead of sales**
Innovative Medicines	Sales expected to **grow mid single digit** Core OpInc **revised upwards** from expected to "grow mid to high single digit" to "**grow high single digit**"
Sandoz	Sales expected to **decline low to mid single digit** Core OpInc **revised downwards** from expected to "decline low to mid-teens" to "**decline mid to high teens**"

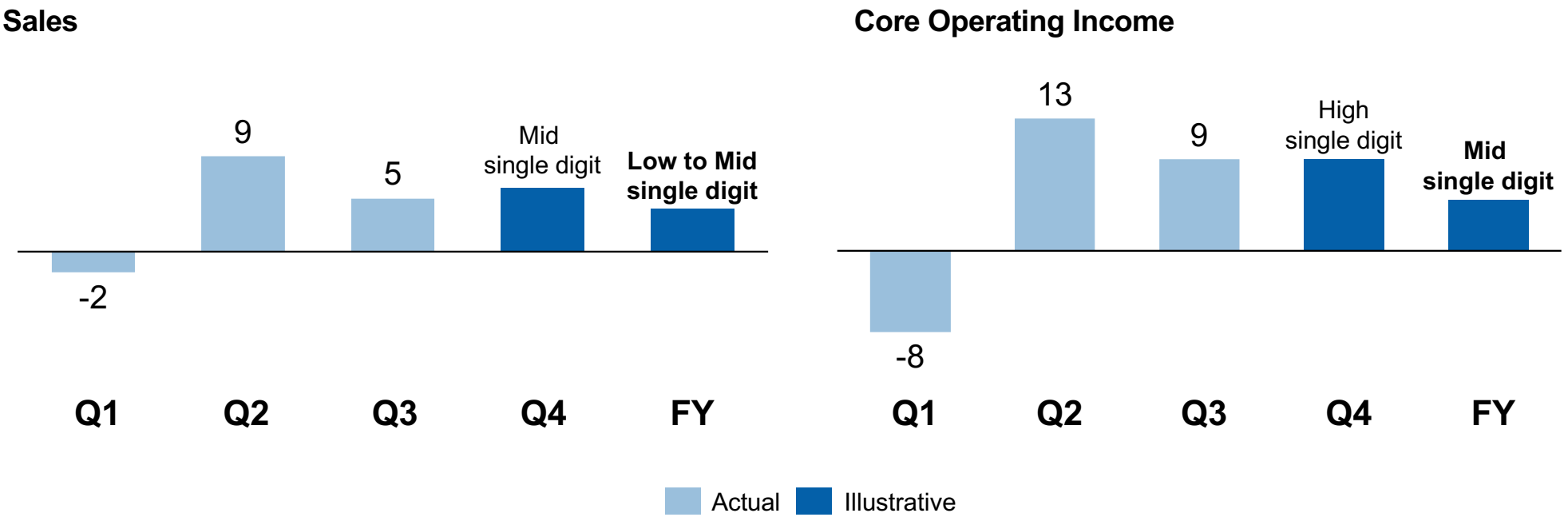
Key assumptions

Continuation of the return to normal global healthcare systems including prescription dynamics, in the remainder of the year.
In addition, we assume that no Gilenya® and no Sandostatin® LAR generics enter in 2021 in the US

We expect Q4 to reflect continuation of return to normal healthcare systems. Q1 / Q2 impacted by PY COVID-19 stocking / destocking

Group quarterly growth vs. PY

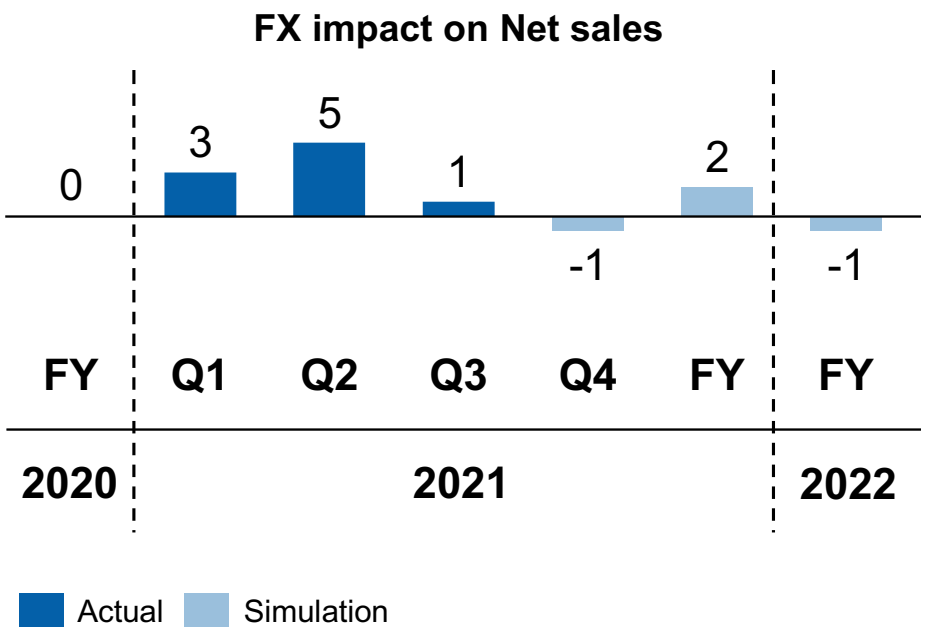
%pts, constant currency

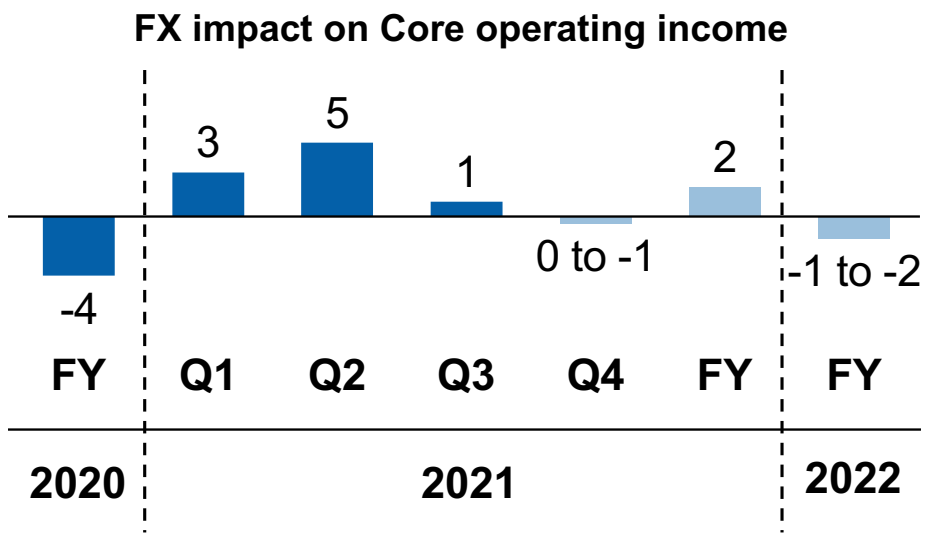


Expected currency impact for full year 2021 and 2022

Currency impact vs. PY

%pts, assuming late-October exchange rates prevail in 2021 and 2022





Vas Narasimhan

Chief Executive Officer

Novartis Capital Markets Day, focus R&D

December 2, 2021
Virtual

Key R&D assets include:

Life-cycle management: Cosentyx® LEQVIO® Power within your reach KISQALI® ribociclib

Mid-stage: Pelacarsen, iptacopan, ligelizumab, remibrutinib, canakinumab, 177Lu-PSMA-617, asciminib

Early-stage: including technology platforms

Meet Novartis Management

May 23-24, 2022
In-person

Consistent long-term performance driving confidence for future

Novartis delivers solid Q3 results, with strong performance of Innovative Medicines

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Raised peak sales guidance for Cosentyx® and Entresto®

We remain confident in the strength of our pipeline and launch brands to fuel the growth of our company in the mid to longer term

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Announced strategic review of Sandoz to maximize shareholder value

Appendix

Strong 9M operational performance from growth drivers

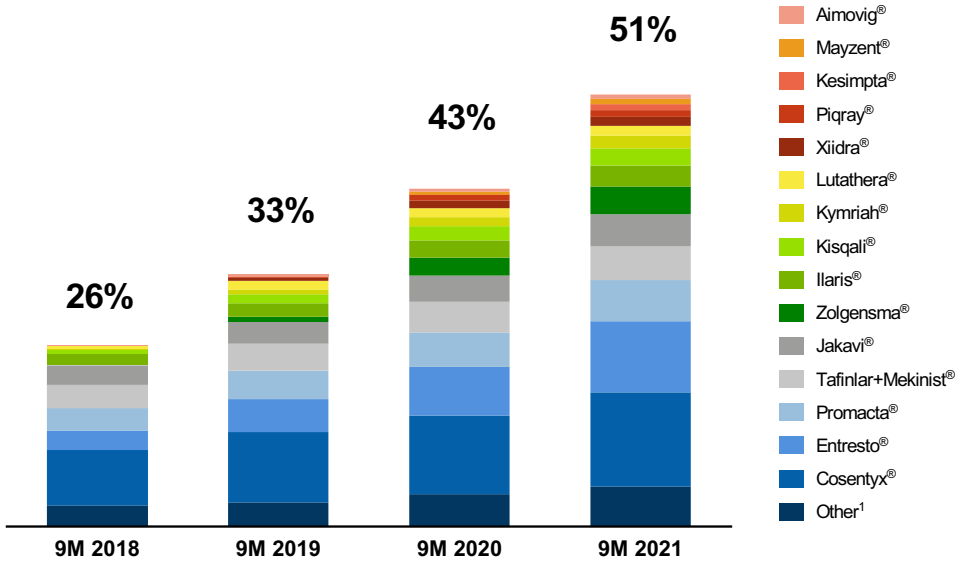
Key growth driver sales 9M 2021[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto® sacubitril/valsartan	2,599	818	41%
Cosentyx® (secukinumab)	3,475	589	18%
zolgensma®	1,009	343	49%
PROMACTA® (eltrombopag)	1,498	231	16%
Kesimpta® (ofatumumab)	225	224	nm
JAKAVI® ruxolitinib	1,187	224	18%
KISQALI® ribociclib	652	149	27%
ILARIS® (canakinumab)	775	142	22%
Xolair® Omalizumab	1,055	139	10%
KYMRIAH® (tisagenlecleucel)	444	111	30%
Tafinlar® + Mekinist®	1,235	101	6%
MAYZENT® (siponimod) tablets	200	87	74%

nm – not meaningful

Driving portfolio rejuvenation

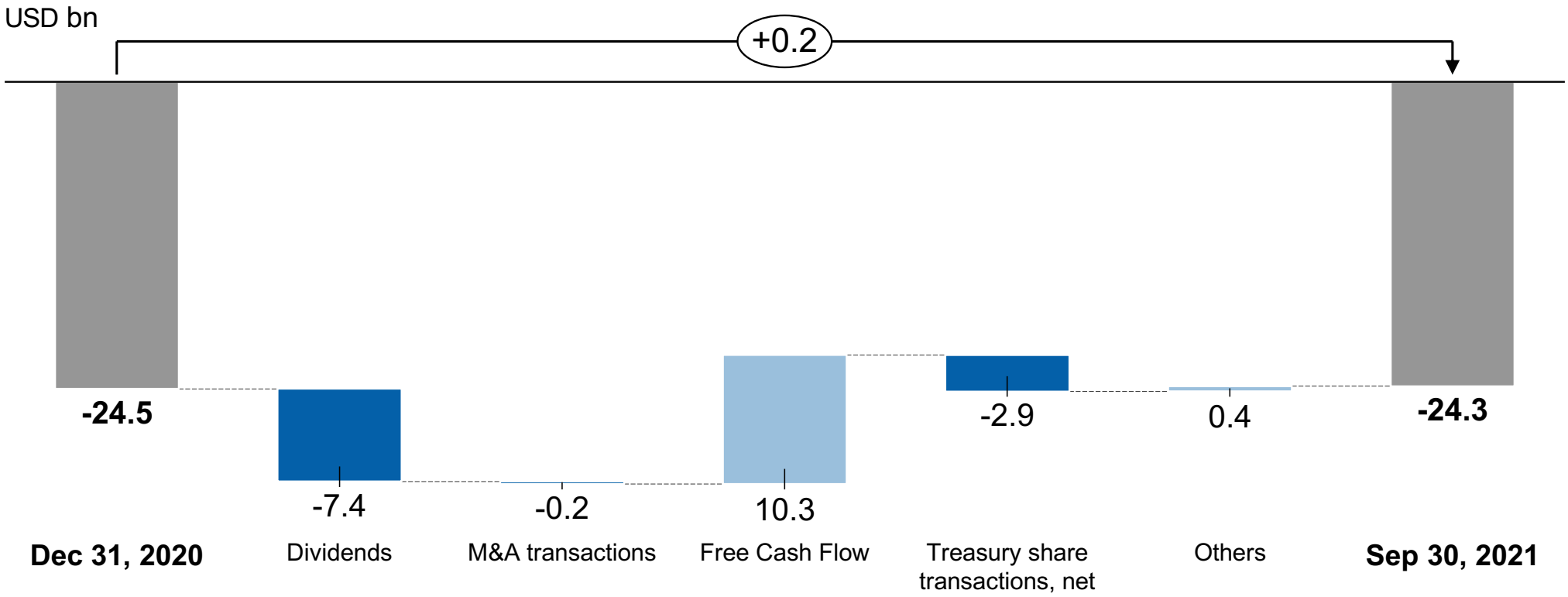
Key growth drivers and launches
51% of IM sales, growing 25% YTD



1. Includes Xolair®, Beovu®, Adakveo®, Tabrecta®, Luxturna®, Enerzair®, Atectura® and Leqvio®

1. Innovative Medicines division. Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 47 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year.

Net debt is broadly in line with December 2020 as FCF was offset by the annual dividend and share buybacks



2021 key pipeline milestones[1]

✓ **Achieved**
✓ **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 (US / EU)	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 MAA submission, evaluation of US BLA submission options ongoing. 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. Q4/2021-Q1/2022 potential COVID impact. 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	**45**	**27**	**6**	**78**
Pharmaceuticals	**55**	**26**	**4**	**85**
Cardiovascular, Renal, Metabolism	**5**	**7**	**1**	**13**
Immunology, Hepatology, Dermatology	**26**	**9**	**2**	**37**
Neuroscience	**4**	**5**	**0**	**9**
Ophthalmology	**5**	**1**	**1**	**7**
Respiratory & Allergy	**8**	**3**	**0**	**11**
Global Health	**7**	**1**	**0**	**8**
Biosimilars	**0**	**2**	**0**	**2**
Total	**100**	**55**	**10**	**165**

Novartis pipeline in Phase 1 (1 of 2)

34 lead indications

Lead indication

Oncology						
Code	**Name**	**Mechanism**	**Indication(s)**			
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors			
ADPT01	ADPT01	-	Colorectal cancer (combos)			
ADPT03	ADPT03	BCL11A	Sickle cell anemia			
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers			
HDM201	HDM201 + MBG453, venetoclax	MDM2 inhibitor	Haematological malignancy			
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			
SQZ622	SQZ622	CD123xCD3 modulator	Acute myeloid leukaemia			
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)	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	r/r DLBCL and r/r adult ALL			

Novartis pipeline in Phase 1 (2 of 2)

34 lead indications

Lead indication

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)	
CEE321	CEE321	Pan JAK inhibitor	Atopic dermatitis	
FIA586	FIA586	-	Non-alcoholic steatohepatitis (NASH)	
MHS552	MHS552	-	Autoimmune indications	
MHV370	MHV370	-	Sjögren's	Systemic lupus erythematosus
NGI226	NGI226	-	Tendinopathy	

Neuroscience

Code	Name	Mechanism	Indication(s)	
LMI070	Branaplam	mRNA splicing modulator	Huntington's disease	
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

24 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)		
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 2L, pediatrics		
BYL719	alpelisib	PI3Kα inhibitor	PIK3CA-related overgrowth spectrum		
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
LXH254	LXH254	cRAF inhibitor	Melanoma (combo)		
MBG453	sabatolimab	TIM3 antagonist	Unfit acute myeloid leukaemia		
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers		
NIS793	NIS793	TGFB1 inhibitor	Colorectal cancer (Combos)		
PDR001	spartalizumab	PD1 inhibitor	Metastatic melanoma (combo)		
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics		
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell anaemia with crisis, pediatrics		

Immunology, Hepatology, Dermatology

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	
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	
MAS825	MAS825	TIM3 inhibitor	NLRC4-GOF indications			
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)		
BLZ945	BLZ945	CSF-1R inhibitor	Amyotrophic lateral sclerosis		
MIJ821	MIJ821	NR2B negative allosteric modulator	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		

1. Clinical hold lifted

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	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 1st line
ACZ885	canakinumab	IL-1b inhibitor	Non-small cell lung cancer (NSCLC), 1L; 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
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 r/r Diffuse large B-cell lymphoma

Immunology, Hepatology, Dermatology

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

1. ^{177}Lu-dotatate in US. 2. Approved in US.

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
			Atypical haemolytic uraemic syndrome
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)

Novartis pipeline in registration

4 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
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 3rd line
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, Hepatology, Dermatology

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

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
KJX839	Leqvio®	siRNA (regulation of LDL-C)	Hyperlipidemia[1]

Ophthalmology

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

1. Approved in EU.

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

LEAD INDICATIONS

2021	2022	2023	2024	≥2025		
177Lu-PSMA-617 Lead AAA617 mCRPC 3L	**ligelizumab** Lead QGE031 CSU	**iptacopan** Lead LNP023 PNH	**JDQ443** Lead JDQ443 2/3L NSCLC (mono)	**^{177}Lu-NeoB** Lead AAA603 Multiple Solid Tumors	**ganaplacide** Lead KAF156 Malaria uncomplicated	**LXH254** Lead Solid tumors (combos)
asciminib Lead ABL001 CML 3L	**sabatolimab**[1] Lead MBG453 HR-MDS		**remibrutinib** Lead LOU064 CSU	**branaplam** Lead LMI070 Huntington's disease	**iscalimab** Lead CFZ533 Sjögren's syndrome	**MIJ821** Lead Depression
tislelizumab Lead VDT482 2L esophageal cancer			**UNR844** Lead Presbyopia	**CEE321** Lead Atopic Dermatitis	**ianalumab** Lead VAY736 Sjögren's syndrome	**NIS793** Lead 1L Pancreatic cancer
			YTB323 Lead 2L r/r Diffuse large B-cell lymphoma	**cipargamin** Lead KAE609 Malaria severe	**icenticaftor** Lead QBW251 COPD	**pelacarsen** Lead TQJ230 CVRR-Lp(a)
				CPK850 Lead RP	**libvatrep** Lead SAF312 COSP	**spartalizumab** Lead PDR001 Metastatic melanoma (combo)
				CSJ117 Lead Asthma	**LNA043** Lead Knee osteoarthritis	**TNO155** Lead Solid tumors
				gevokizumab Lead VPM087 1st line CRC / 1st line RCC	**LXE408** Lead Visceral leishmaniasis	**tropifexor&licogliflozi** Lead LJN452 NASH (combos)

NEW INDICATIONS

tislelizumab LCM VDT482 1L Nasopharyngeal Carcinoma	**177Lu-PSMA-617** LCM AAA617 Pre-taxane	**tislelizumab** LCM VDT482 1L ESCC	**177Lu-PSMA-617** LCM AAA617 mHSPC	**asciminib** LCM ABL001 CML 1L	**iptacopan** LCM LNP023 aHUS	**ligelizumab** LCM QGE031 CINDU
tislelizumab LCM VDT482 NSCLC	**iptacopan** LCM LNP023 C3G	**tislelizumab** LCM VDT482 Localized ESCC	**sabatolimab** LCM MBG453 Unfit AML	**asciminib** LCM ABL001 CML, 2L, pediatrics	**iptacopan** LCM LNP023 iMN	**remibrutinib** LCM LOU064 Sjögren's syndrome
	iptacopan LCM LNP023 IgAN	**tislelizumab** LCM VDT482 1L Hepatocellular Carcinoma	**tislelizumab** LCM VDT482 1L Small Cell Lung Cancer	**cipargamin** LCM KAE609 Malaria uncomplicated	**iscalimab** LCM CFZ533 Liver Tx	**remibrutinib** LCM LOU064 Multiple sclerosis
	tislelizumab LCM VDT482 1L Gastric Cancer		**tislelizumab** LCM VDT482 1L Bladder Urothelial Cell Carcinoma	**ianalumab** LCM VAY736 AIH	**ligelizumab** LCM QGE031 Food allergy	

1. 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

2021	2022	2023	2024	≥2025		
alpelisib LCM BYL719 PROS	**Cosentyx** LCM secukinumab, AIN457 PsA IVIV	**canakinumab** LCM ACZ885 Adjuvant NSCLC	**Adakveo** LCM SEG101 Sickle cell anaemia with crisis ped	**Atectura** LCM indacaterol + mometasone, QMF149 Asthma, pediatrics	**Cosentyx** LCM secukinumab, AIN457 Lichen Planus	**Mayzent**[4] LCM siponimod, BAF312 Multiple sclerosis, pediatrics
Beovu LCM brolucizumab, RTH258 DME	**Cosentyx** LCM secukinumab, AIN457 AS H2H	**Cosentyx** LCM secukinumab, AIN457 AS IVIV	**Coartem** LCM artemether + lumefantrine, COA566 Malaria uncompl., formula for <5kg	**Aimovig** LCM erenumab, AMG334 Pediatric Migraine	**Jakavi** LCM ruxolitinib, INC424 Myelofibrosis (combination)	**Piqray** LCM alpelisib, BYL719 HER2+ adv BC
Cosentyx LCM secukinumab, AIN457 Juvenile idiopathic arthritis	**Cosentyx** LCM secukinumab, AIN457 Hidradenitis suppurativa	**denosumab** BioS GP2411 anti RANKL mAb	**Cosentyx** LCM secukinumab, AIN457 GCA	**aflibercept** BioS SOK583 Neovascular age-related macular degeneration	**Kesimpta**[3] LCM ofatumumab Multiple sclerosis, pediatrics	**Rydapt** LCM midostaurin, PKC412 Acute myeloid leukemia, pediatrics
Jakavi LCM ruxolitinib, INC424 Chronic GVHD	**Entresto EU**[1] LCM sacubitril/valsartan, LCZ696 Pediatric CHF	**Kisqali** LCM ribociclib, LEE011 HR+/HER2- BC (adj)	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Acute GVHD	**Beovu** LCM brolucizumab, RTH258 Diabetic retinopathy	**Kymriah** LCM tisagenlecleucel, CTL019 1L high risk ALL, pediatrics & young adults	**Zolgensma** LCM AVXS-101 OAV101 SMA IT
Jakavi LCM ruxolitinib, INC424 Acute GVHD	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 HGG/LGG - Pediatrics	**Lutathera** LCM ^{177}Lu-oxodotreotide[2] GEP-NET 1L G3	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Chronic GVHD	**Cosentyx** LCM secukinumab, AIN457 Lupus Nephritis	**Leqvio** LCM KJX839 CVRR-LDLC	
Kymriah LCM tisagenlecleucel, CTL019 r/r Follicular lymphoma	**Xolair** LCM omalizumab, IGE025 Auto-injector	**Piqray** LCM alpelisib, BYL719 TNBC	**Leqvio** LCM KJX839 Ped Hyoerlipidemia			
		Piqray LCM alpelisib, BYL719 Ovarian cancer	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 Thyroid cancer			
		Promacta LCM eltrombopag, ETB115 r/r severe aplastic anemia				
		Xolair LCM omalizumab, IGE025 Food allergy				

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.novartisclinicaltrials.com

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 Milesstone(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)	2022
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 Milesstone(s)	2023
Publication	TBD

Leqvio® - Inhibitor

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 Milesstone(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 9, 10 & 11 studies)
Read-out Milesstone(s)	2022	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 Milesstone(s)	Primary: Q3-2020 (actual); Final: H2-2021	2023
Publication	TBD	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT04659863 ORION-13 (CKJX839C12302)
Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	15
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330
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.
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milesstone(s)	2023
Publication	TBD

iptacopan - CFB inhibitor

Study	NCT03832114 (CLNP023X2202)	NCT03955445 (CLNP023B12001B)
Indication	C3 glomerulopathy (C3G)	C3 glomerulopathy (C3G)
Phase	Phase 2	Phase 2
Patients	27	27
Primary Outcome Measures	Cohort A: Ratio to Baseline of UPCR to Week 12 derived from 24hr urine collection Cohort B: Change from Baseline in C3 Deposit Score (based on immunofluorescence microscopy) at Week 12	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	Increasing doses of LNP023 up to 200mg bid: Cohort A: Native kidney patients Cohort B: Kidney transplanted patients	Open-label LNP023 200mg bid
Target Patients	Patients with C3 glomerulopathy	Patients with C3 glomerulopathy
Read-out Milesstone(s)	H1-2021 (actual)	2025
Publication	Actual: Interim analysis data from Cohort-A presented at American Society of Nephrology (ASN 2020). Planned: Note not to be communicated externally until accepted. 1) Planned abstract at ERA-EDTA, Q3 2021 2) Planned abstract at ASN, Q4 2021	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 Milesstone(s)	2023	2023
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

iptacopan - CFB inhibitor

Study	NCT04817618 APPEAR-C3G (CLNP023B12301)	NCT04889430 APPELHUS (CLNP023F12301)
Indication	C3 glomerulopathy	Atypical haemolytic uraemic syndrome
Phase	Phase 3	Phase 3
Patients	68	50
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)	Percentage of participants with complete TMA response without the use of PE/PI and anti-C5 antibody
Arms Intervention	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.	Single arm open-label with 50 adult patients receiving 200mg oral twice daily doses of iptacopan
Target Patients	Patients with native C3G	Adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody)
Read-out Milesstone(s)	2023	2024
Publication	TBD	TBD

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 Milesstone(s)	2024
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 Milesstone(s)	2022	Primary 2024
Publication	TBD	TBD

Cosentyx® - IL-17A inhibitor

Study	NCT03031782 (CAIN457F2304)	NCT03259074 SURPASS (CAIN457K2340)
Indication	Psoriatic arthritis	Ankylosing spondylitis
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 Milesstone(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 Milesstone(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 Milesstone(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 Milesstone(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 Milesstone(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 Milesstone(s)	Primary 2023 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 Milesstone(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 Milesstone(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 Milesstone(s)	Primary completion date: 2024
Publication	

ligelizumab - IgE inhibitor

Study	NCT02649218 (CQGE031C2201E1)	NCT03580356 Pearl 2 (CQGE031C2303)
Indication	Chronic spontaneous urticaria / Chronic idiopathic urticaria	Chronic spontaneous urticarial / Chronic idiopathic urticaria?
Phase	Phase 2	Phase 3
Patients	226	1050
Primary Outcome Measures	Long-term safety; number of participants with treatment-emergent adverse events	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12
Arms Intervention	Ligelizumab 240 mg q4wks open label for 52 weeks	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	Adult patients with chronic spontaneous urticaria inadequately controlled with H1-antihistamines at approved or increased doses, alone or in combination with H2-antihistamines or leukotriene receptor antagonists.	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines
Read-out Milesstone(s)	2019 (actual)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)
Publication	Manuscript: Primary results extension trial (Allergy), H2 2021	Past publications: Study design presented at UCARE 2018 Congress: primary results EADV 2022 (H2 2022) or AAAAI 2023 Manuscript: primary results, Journal (TBD), 2023

ligelizumab - IgE inhibitor

Study	NCT03580369 Pearl 1 (CQGE031C2302)	NCT04210843 (CQGE031C2302E1)
Indication	Chronic spontaneous urticaria	Chronic spontaneous urticaria
Phase	Phase 3	Phase 3
Patients	1050	800
Primary Outcome Measures	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12	The proportion of subjects with well-controlled disease (UAS7 ? 6) 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 1 and 3 Ligelizumab Dose 2 and 3
Target Patients	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines	Patients who completed studies CQGE031C2302, CQGE031C2303, CQGE031C2202 or CQGE031C1301
Read-out Milesstone(s)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)	2026
Publication	Past publications: Study design presented at UCARE 2018 Congress: primary results EADV 2022 (H2 2022) or AAAAI 2023 Manuscript: primary results, Journal (TBD), 2023	Study design presented at 2020 EAACI

remibrutinib - BTK inhibitor

Study	NCT03926611 (CLOU064A2201)	NCT04109313 (CLOU064A2201E1)
Indication	Chronic spontaneous urticaria (CSU)	Chronic spontaneous urticaria (CSU)
Phase	Phase 2	Phase 2
Patients	308	250
Primary Outcome Measures	Change from baseline in weekly Urticaria Activity Score (UAS7) at Week 4	Long-term safety and tolerability
Arms Intervention	Arm 1 Low dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 2 Medium dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 3 High dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 4 Low dose of LOU064 orally, twice daily from Day 1 to 85 Arm 5 Medium dose of LOU064 orally, twice daily from Day 1 to 85_x0	Selected dose of LOU064 taken orally twice a day (morning and evening) from day 1 to week 52
Target Patients	Adults with CSU inadequately controlled by H1-antihistamines	Patients with CSU who have participated in preceding studies with LOU064
Read-out Milesstone(s)	H2-2021 (actual)	2022
Publication	H2-2021 (EADV)	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 Milesstone(s)	2023
Publication	2023

Neuroscience

MIJ821- NR2B negative allosteric modulator (NAM)

Study	NCT04722666 (CMIJ821A12201)
Indication	Major depressive disorder
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 for
Target Patients	Participants who have suicidal ideation with intent
Read-out Milesstone(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 Milesstone(s)	Double-blind FIR for 100% of pts 2021; Q4 2021 Extension (open-label): 2024
Publication	Planned in H2-2022 for double-blind phase and H1-2025 for open-label extension phase

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 Milesstone(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 Milesstone(s)	2026
Publication	TBD

Zolgensma® - Gene Therapy

Study	NCT03505099 SPR1NT (COAV101A12303)	NCT03837184 STRIVE Asia Pacific (COAV101A12304)
Indication	Spinal muscular atrophy	Type 1 spinal muscular atrophy
Phase	Phase 3	Phase 3
Patients	30	2
Primary Outcome Measures	[2 copies of SMN2] Percentage of participants achieving functional independent sitting for at least 30 seconds at any visit [3 copies of SMN2] Percentage of participants achieving the ability to stand without support for at least 3 seconds at any visit	Proportion of participants sitting without support
Arms Intervention	Open-label, single-arm, single-dose, intravenous	Open-label, single-arm, single-dose, intravenous
Target Patients	Pre-symptomatic patients with spinal muscular atrophy and multiple copies SMN2	Patients with spinal muscular atrophy Type 1
Read-out Milesstone(s)	H2-2021 (3-copy cohort)	H2-2021
Publication	Final study results of two-copy cohort were presented as late-breaker oral presentation at EAN Jun 22 2021; final study results of three-copy cohort to be presented at 2022 congress.	TBD

Zolgensma® - SMN1 gene replacement therapy

Study	NCT03381729 STRONG (COAV101A12102)
Indication	Type 2 spinal muscular atrophy
Phase	Phase 1
Patients	51
Primary Outcome Measures	Safety and tolerability, incidence of adverse events Proportion of patients achieving Standing Milestone Change in Hammersmith Functional Motor Scale
Arms Intervention	Open-label, single-arm, single-dose, intrathecal
Target Patients	Patients with spinal muscular atrophy with 3 copies of SMN2
Read-out Milesstone(s)	Cohort B: Q4-2019 (actual); Cohort C1: TBC
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 Milesstone(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	TBD

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 Milesstone(s)	2018 (actual)	2022
Publication	Manuscript planned for submission H2-2021	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 Milesstone(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 Milesstone(s)	Primary: Q4-2020 (actual); Final: Q4-2021	Primary: Q3-2020 (actual); Final: Q3-2021 (actual).
Publication	Brown et al., presented at ARVO May 2021 Manuscript submission H2 2021	Brown et al., presented at ARVO May 2021 Manuscript submission H2 2021

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 Milesstone(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	Arm 1: RTH258 (Brolucizumab) 6 mg3 x q6w loading injections, followed by q12w maintenance through week 90 Arm 2: Panretinal photocoagulation laser initial treatment in 1-4 sessions up to Week 12, followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Read-out Milesstone(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 Milesstone(s)	2023
Publication	2023

Respiratory & Allergy

CSJ117 - 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 Milesstone(s)	2022
Publication	Primary publications planned for 2022

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 Milesstone(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 Milesstone(s)	Primary Analysis: H1-2023
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 Milesstone(s)	H2-2021
Publication	Johnson B et al. Presented at AACR-NCI-EORTC 2019 (safety run-in) Planned abstract submission to congress in 2H 2021

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 Milesstone(s)	2023
Publication	TBD

NIS793 - TGFβ1 inhibitor

Study	**NCT04935359 (CNIS793B12301)**
Indication	1L Pancreatic cancer
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 Milesstone(s)	2026
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 Milesstone(s)	2023	2022
Publication	TBD	TBD

177Lu-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 Milesstone(s)	Primary Analysis: 2022 Final Analysis: 2025	Primary Analysis: 2024
Publication	TBD	TBD

Kisqali® - 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 Milesstone(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 Milesstone(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 Milesstone(s)	2023
Publication	TBD

Tabrecta® - 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 Milesstone(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 Milesstone(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 Milesstone(s)	2022
Publication	TBD

Hematology

Adakveo® - 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 Milesstone(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 Milesstone(s)	H2-2021 (pediatric patients >=12 year old) 2024 (pediatric patients <12 year old)
Publication	Abstract submission to ASH 2021

Jakavi® - 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	42
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 Milesstone(s)	2023	2023
Publication	TBD	TBD

Jakavi® - 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 Milesstone(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 Milesstone(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	60	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 Milesstone(s)	Primary CSR: 2022 Final CSR: 2025	Primary: 2021; Final: 2023
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 Milesstone(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 Milesstone(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 Milesstone(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 Milesstone(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)

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 Milesstone(s)	2022-2023	2023
Publication		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 Milesstone(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 Milesstone(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 Milesstone(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 - PGH-1

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 Milesstone(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)	2022
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

References

Top-line results for CANOPY-1

1 Novartis Data on File

2 ClinicalTrials.gov. Brief Title: Study of Efficacy and Safety of Canakinumab as Adjuvant Therapy in Adult Subjects With Stages AJCC/UICC v. 8 II-IIIA and IIIB (T>5cm N2) Completely Resected Non-small Cell Lung Cancer Acronym: CANOPY-A (Canopy-A). Available at: https://www.clinicaltrials.gov/ct2/show/NCT03447769. Accessed on September 10, 2021.

3 Ridker PM, Thuren T, Zalewski A, et al. Interleukin-1β inhibition and the prevention of recurrent cardiovascular events: rationale and design of the Canakinumab Anti-inflammatory Thrombosis Outcomes Study (CANTOS). Am Heart J. 2011;162:597–605.

4 ClinicalTrials.gov. Study of Efficacy and Safety of Pembrolizumab Plus Platinum-based Doublet Chemotherapy With or Without Canakinumab in Previously Untreated Locally Advanced or Metastatic Non-squamous and Squamous NSCLC Subjects (CANOPY-1). Available at: https://clinicaltrials.gov/ct2/show/NCT03631199. Accessed on September 10, 2021.

5 ClinicalTrials.gov. Gevokizumab With Standard of Care Anti-cancer Therapies for Metastatic Colorectal, Gastroesophageal, and Renal Cancers. Available at: https://clinicaltrials.gov/ct2/show/NCT03798626. Accessed September 10, 2021

6 Jayaraman. P. Targeting IL-1β pathway for cancer immunotherapy. Proceedings of the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics; 2019 Oct 26-30

Cosentyx® peak sales expectations

1 Market defined as US + EU5 Biologics in PsO, PsA and AS. Source: US: IQVIA PADDS Monthly Jul'21 and Indication split% from SHS PTD for disease% for 2018 used for PsA, AS and IQVIA Medical for PSO. EU5- IQVIA Monthly Jul'21, CPO Inputs Jun'21 and IQVIA Padds Jul'21

2 PSO: DRG + IQVIA patient equivalents. SpA: Aligned with DRG and multiple internal sources

Entresto® grew +44% in the quarter

1 2021 Update to the 2017 ACC Expert Consensus Decision Pathway (ECDP) for Optimization of Heart Failure Treatment: Answers to 10 Pivotal Issues About Heart Failure With Reduced Ejection Fraction. https://www.jacc.org/doi/10.1016/j.jacc.2020.11.022

2 2021 ESC Guidelines for the diagnosis and treatment of acute and chronic heart failure https://doi.org/10.1093/eurheartj/ehab368

3 IQVIA National Prescription Audit as of 24/09/2021

Entresto® peak sales expectations

1 Eligible patients defined as prevalent HFrEF patients within each market's label. G7 = US, CA, JP, DE, FR, IT, UK

2 Including but not limited to CA, SP, UK

Leqvio® on track for US launch

1 In patients with elevated LDL-C despite treatment with maximally tolerated statin therapy. V-INITIATE NCT04929249; V-INCEPTION NCT04873934