Investigator-Initiated Trials / Studies »

As part of our commitment to delivering innovative therapies to patients worldwide, Novartis believes in the need to support ethical independent clinical research conducted by qualified third-party investigators.

The value of the scientific research produced by these investigators is key to complementing Novartis-sponsored research by helping to ensure we better understand the benefit/risk profile of our therapies, as well as enabling us to explore new opportunities addressing unmet medical needs.

The proposed clinical research must offer meaningful scientific and/or clinical objectives supported by valid study designs in which the privacy rights, safety and welfare of patients is of paramount importance.

Novartis defines IITs as â€œstudies with scientific and medical merit developed and sponsored by an independent investigator or academic sponsor. An IIT may be a clinical or non-clinical study conducted without the participation of Novartis, for which the IIT sponsor requests Novartis to provide either funding, drug product or both.â€

Novartis Position on Investigator Initiated Trials (IITs) and Investigator Initiated Research (IIRs)
(PDF 0.2 MB)

IIT guidance for investigators (PDF 0.7 MB)

Strategic areas of interest

We welcome unsolicited research proposals from qualified investigators in our strategic areas of interest which we list below. Well-thought through studies that enhance our delivery of innovative therapies to more patients worldwide, enhance patient care, and align with our strategic areas of interest will be considered. If you have questions on any steps of the process or wish to discuss your study concept, please feel free reach out to your local Novartis contact (e.g. MSL, Medical Advisor) for support.

COVID-19: We are interested in COVID-19 vaccination studies in combination with Novartis products.

Cardiovascular, Renal & Metabolism

Inclisiran (Leqvio®)

Studies within the label population

- Long-term safety and tolerability
- Health-related quality of life (HrQol)
- Implementation science and/or quality system improvement programs (ex. clinical care pathways)
- Studies on apheresis in HeFH

Mechanistic Studies in secondary prevention

- Remodeling, fibrosis, inflammation
- Plaque burden regression/modification
- CABG graft remodeling

Mechanistic Studies in primary prevention and/or statins intolerant
- Remodeling, fibrosis, inflammation

Assessment techniques (IVUS, EKO, CCTA) must be guidelines validated (pending vascular bed assessment)

Out of scope:

- Studies in off-label populations (with respect to geographies)
- Efficacy, safety and tolerability studies with inclisiran in pediatric population (<18 y)
- Studies in different populations than ASCVD, ASCVD equivalent, and FH
- CVOT trials
- Pre-Clinical Proposals (separate process)

Iptacopan (LNP023)*

With Drug

1. Mechanistic studies in IgAN, C3G, aHUS
2. Subgroups of patients that are included in the overall study population in indications pursued with iptacopan (IgAN, C3G, aHUS)

Without Drug

1. Role of complement system in complement-mediated kidney diseases
2. Additional ways to foster diagnosis of glomerulopathies beyond biopsy
3. Studies which attempt to clarify the histopathologic complexity/equipoise of C3G
4. Identification of approaches that lead to better characterization, management or correlation with outcomes in IgAN, C3G, aHUS, MN, LN â€“ e.g. identification of biomarkers, genetic analysis or biopsy-based studies
5. Burden of disease (clinical, economic, and/or humanistic burden) - IgAN, C3G, aHUS, MN, LN
6. Epidemiology studies (incl. registries) - IgAN, C3G, aHUS, MN, LN

Out of scope:

- Pediatric studies (with drug)
- Studies exploring different dosing regimens as currently investigated
- Any study, which combines iptacopan with immunosuppressant
- Head-to-head comparisons
- Studies including patients with CKD stages 4 and 5

* Strategic areas of interest for iptacopan (PNH), please also refer to the Oncology section

Sacubitril/Valsartan (Entresto®)

Studies on improvements of HF care through increase in GDMT

Studies with sac/val in Chronic Heart Failure with reduced EF:

- Safety and tolerability in populations not well represented in PARADIGM-HF, PIONEER-HF
  - De-novo heart failure
  - ACEi/ARB naive

Mechanistic Studies in HF looking at

- Remodeling, fibrosis, inflammation
- Cardiac function (including diastolic function)
- Cardiac biomarkers

Population with specific, less well studied / documented HF etiologies, e.g. chemotherapy /toxicity induced HF

Out of scope:
- Studies in HFpEF (EF > 60%) or post-MI patients
- Comparative effectiveness studies vs other MoA, e.g. SGLT2i
- Studies in non-cardiovascular disease
- Studies in patients with valvular disorders not related to HF
- Studies focused on hypertension, including resistant hypertension, in geographies where it is not in-label
- Studies in children (<18 years)

Gene Therapies

Zolgensma

Indication: Spinal Muscular Atrophy (SMA)

- Biomarkers
- Methods/Processes to reduce time to diagnosis & speed to treatment
- Expansion of treatment access for our gene therapy
- Demonstrating or validating care needs for SMA populations post-gene therapy treatment
- Value of gene therapy: cost of care, quality of life, and caregiver burden

Immunology

Canakinumab (Ilaris)

- Registries with data on Familial Mediterranean Fever (FMF):
  - Real-life data on colchicine resistance, lack of tolerance and suboptimal response to colchicine (retrospective)
  - Impact on quality of life (QoL) of suboptimal response to colchicine; impact seen by patients vs that by HCPs (prospective)
- Clinical studies looking at the window of opportunity in Still’s disease:
  - Early use of canakinumab in Still’s disease
- Studies in rare systemic autoinflammatory diseases (SAID):
  - Schnitzler Syndrome
  - Periodic Fever, Aphthous stomatitis, Pharyngitis and cervical Adenitis (PFAPA)
  - Pyogenic sterile Arthritis Pyoderma gangrenosum and Acne (PAPA)
  - Chronic Recurrent Multifocal Osteomyelitis (CRMO)
  - Kawasaki
  - Behçet disease
  - Yao syndrome
  - Post-hoc analyses of existing trials
- Biomarkers in Still’s disease & IL-1 signature
- Out of scope
  - Osteoarthritis & other high prevalence indications
  - Pre-clinical IITs

Secukinumab (Cosentyx)

Indications: Psoriasis (PsO), Psoriatic Arthritis (PsA), Axial SpA (ankylosing spondylitis, nr-axSpA)
• Studies assessing:
  ○ Clinical outcomes addressing one or several key clinical manifestations of PsA, peripheral SpA or axial manifestations with axial SpA
  ○ Novel imaging techniques targeting axial or peripheral manifestations (synovitis, enthesitis), structural progression, repair process and bone remodelling in peripheral and axial SpA
  ○ Implementation of early diagnosis and management of psoriatic disease or SpA to improve long term outcomes
• Exploratory studies assessing:
  ○ Treatment effects on selected systemic manifestations and/or comorbidities of psoriatic disease or SpA
  ○ Novel imaging techniques to investigate the role of secukinumab in limiting progression from PsO to PsA and nr-axSpA to Axial SpA
  ○ Machine learning techniques to create predictive models for disease trajectories, and IL-17A inhibition responses across disease phenotypes/genotypes
• Mechanistic studies assessing:
  ○ Early treatment and disease modification, use of biomarkers to predict disease and treatment outcomes
  ○ Roles of different pathways (TNFa, IL-17, IL-22/IL-23) in enthesitis, axial disease, dactylitis and peripheral SpA
  ○ Role of IL 17 pathways in different clinical manifestations of lupus nephritis, Giant cell arteritis, ERA-JIA and JPsA
  ○ Pathways in diseases with potential role of IL-17A e.g. Hidradenitis Suppurativa, Lichen Planus, Ichthyosis/Netherton syndrome, Pityriasis Rubra Pilaris, non-ocular Bechetâ€™s Disease, Papulopustular Rosacea, JIA uveitis, CRMO/SAPHO, or others
• RWE studies assessing:
  ○ Effectiveness, drug survival, cost-effectiveness, resource utilization and treatment patterns across approved indications
  ○ T2T or guidelines implementation strategies to achieve disease remission
• Out of scope:
  ○ Combination studies for secukinumab with other biologic agents
  ○ Studies in: safety topics e.g. infections (tuberculosis, HIV, viral hepatitis), transplant, sarcoidosis, high-risk patients
  ○ Life-threatening conditions (evaluated on a case-by-case basis)
  ○ Studies related to unapproved indications (evaluated on a case-by-case basis)

**Neuroscience**

**Ofatumumab (Kesimpta) and siponimod (Mayzent) - Multiple sclerosis (MS)**

• Focus on prognosis and diligent monitoring of patients with MS (including data and digital):
  ○ Markers for disease prognosis, disease monitoring, and/or risk mitigation
  ○ New or improved quantitative outcome measures in MS, including next-generation technology and patient assessment technologies
  ○ Integration of markers/outcome measures to establish disease stability or disease control, disease progression
• Mechanistic studies looking at differentiating Novartis compounds from other DMTs

**Oncology**

**Piqray / Vijoice (Alpelisib)**
BREAST CANCER

- HR+ / HER2 - studies in breast cancer patients exploring:
  - Post adjuvant CDK4/6 inhibitors + endocrine therapy
  - Patient care, safety and tolerability related studies (e.g., AEs with special interest, Quality of Life, Digital health solution)
- Studies in advanced breast cancer an ovarian indications:
  - Combination of Alpelisib with other compounds in view of the evolving landscape
  - Differentiation from established/future compounds, clinically relevant patients groups, etc

RARE

- Alpelisib in non-cancer pathologies with evidence of a PIK3CA mutation and/or an activation of the PI3K/AKT/mTOR pathway

Out of scope:

- BREAST CANCER - Any area outside of advanced breast and ovarian cancer
- RARE - Alpelisib in PROS

Asciminib (Scemblix)

Studies in the label population (adult patients with Ph+ CML-CP, previously treated with two or more tyrosine kinase inhibitors):

- Clinical efficacy and safety in real-world setting
- Long-term safety and tolerability
- Treatment optimization in 3rd line

Studies exploring additional patient populations in CML:

- Use of asciminib in earlier treatment lines, such as 2\textsuperscript{nd} line and newly diagnosed CML
- Treatment Free Remission
- High need populations such as Ph+ALL, CML-AP/BCÂ
- Use of combinations

Studies providing insight into mechanistical action of asciminib, potential on- and off target effects and its use against additional mutations in patients with CML.

Out of scope:

- Use of asciminib in ABL-independent diseases

Canakinumab (ACZ885)

Non small cell lung cancer

- Anti-IL-1\textsuperscript{Î²} in oncology as a MoA: Neo/Adjuvant treatmentÂ in combinationsÂ with IO and/orÂ radiotherapy; lung cancer preventionÂ (including MRD ,ctDNA)
- PatientÂ selection: predictive and/orÂ prognosticÂ biomarkers,Â inflammation-related biomarkers, Tumor phenotype
- TheÂ role of inflammasome pathway in thoracic malignancies
Out of scope:
- Interventional studies in advanced/metastatic NSCLC
- Any study that overlaps with current Novartis study
- Alternative dosing

Iptacopan (LNP023)**

With Drug
1. Mechanistic studies in Paroxysmal Nocturnal Hemoglobinuria (PNH);
2. Studies evaluating factors associated with or predictive of treatment outcome in PNH;
3. Studies exploring preferences in oral treatment administration approaches in PNH

Without Drug
1. Role of complement system in complement-mediated PNH, Immune Thrombocytopenia Purpura (ITP) and Cold Agglutinin Disease (CAD);
2. Approaches to facilitating and expediting diagnosis of PNH and CAD;
3. Identification of biomarkers that leads to better characterization, management or correlation with outcomes in PNH, ITP and CAD;
4. Burden of disease (clinical, economic, and/or humanistic burden) â€“ PNH and CAD;
5. Epidemiology studies (incl. registries) â€“ PNH and CAD

Out of scope:
- Pediatric studies
- Studies exploring different dosing regimens as currently investigated
- Any study, which combines iptacopan with immunosuppressant and anti-C5 treatments
- Head-to-head comparisons
- Studies in other hematology diseases

**Â Strategic areas of interest for iptacopan (IgAN, C3G, aHUS, MN, LN), please also refer to the Cardiovascular, Renal & Metabolism section

JDQ443
- Translational research on determinants and mechanisms of response and/or resistance to JDQ443
- Combinations of JDQ443:
  - 1L or 2L combinations
  - 2L combination with chemotherapy
- JDQ443 in early-stage resectable NSCLC (neoadjuvant and/or adjuvant)
- Efficacy of 2L JDQ443 monotherapy vs. other 2L line combinations (e.g. chemo plus VEGFi)
- JDQ443 combinations in KRAS\(^{G12C}\) inhibitor-pretreated, refractory patients
- JDQ443 in other KRAS G12C mutated tumours (e.g. GI or GYN)

Out of scope:
- H2H interventional studies versus other \(KRAS G12C\) inhibitors
- Any study in overlap with ongoing Novartis-sponsored/supported studies

[177Lu] Lu-DOTA-TATE (LutatheraÂ®)
• Studies with Lutathera in well differentiated SSTR+ tumors of neuroendocrine origin
• Retrospective studies with additional cycles of Lutathera in SSTR+ GEP-NET patients after having progressed on initial Lutathera treatment (re-treatment/re-challenge)
• Retrospective studies describing treatment sequence in metastatic GEP-NET patients
• Studies with Lutathera in metastatic NET patients in combination with other anti-cancer treatments, including chemotherapy, immuno-oncology therapies, tyrosine kinase inhibitors, PARP-inhibitors, CDK4/6 inhibitors or other upcoming treatments (if supported by MoA rationale)
• Studies describing the Quality of Life/ Patient reported outcomes related to Lutathera treatment
• Retrospective studies describing long-term safety or health economic aspects
• Studies with neoadjuvant Lutathera use in GEP-NET
• Intraarterial Lutathera administration studies in GEP-NET

[177Lu] Lu-PSMA-617

• Focus on 1/2L mCRPC and nm (localized, loco-regional) and mHSPC (De Novo and Relapse)
  ○ Chemo-unfit /frail patients population
  ○ Head to head vs chemotherapy in 1L mHSPC
  ○ Combination and sequencing strategies
    ■ with PSMA enhancers / sensitizers in mHSPC
    ■ to reduce and control AE management
    ■ with PARP inhibitor or Immunotherapeutic in mHSPC
    ○ Pattern of treatment sequencing to understand potential cross-resistance
    ○ Re-treatment strategies from localized, loco-regional or mHSPC to mCRPC
    ○ Long-term safety studies and/or post-progression evaluations
    ○ Burden of illness and quality of life studies
• Biomarker / translational studies
  ○ Predictive to efficacy and safety, as well as disease aggressiveness
  ○ Informative (PSMA-PET)
  ○ Resistance
  ○ Regulation of PSMA expression in different disease stages
• Out of scope:
  ○ all other tumor types beyond prostate cancer
  ○ Clinical studies in COVID-19

Ribociclib (Kisqali)

• HR+/HER2- studies in breast cancer patients: Exploring strategies to inform treatment sequencing across early to advanced breast cancer
• HR+/HER2- studies in breast cancer patients: Utilizing real world data and/or digital health technologies
• HR+/HER2- studies in breast cancer patients: Utilizing patient reported outcomes (PRO)
• Mechanistic studies looking at CDK4 and CDK6 function in cellular senescence, immunomodulation, and endocrine resistance

Out of scope:

• Any topic outside HR+/HER2- breast cancer

Sabatolimab (MBG453)

• Biomarker studies (e.g. TIM-3, Leukemic Stem Cell, etc.)
• Myelodysplastic Syndrome (MDS) and Acute Myeloid Leukemia (AML): novel-novel combinations,
optimal sequence
• Burden of Illness and Quality of Life studies in MDS and AML
• Pattern of treatment (incl. transplant), Real world (RW) agents treatment in MDS and AML

Out of scope:
• Solid tumors and other non-hematology indications
• Pediatric studies
• Clinical studies in COVID-19

Tisagenlecleucel (Kymriah)
• Identification (± modification) of factors influencing clinical outcomes
• New combinations and optimized treatment sequencing
• Strategies for overcoming resistance and relapse
• Optimization of patient management to reduce toxicity
• Demonstrating effectiveness, safety and resource utilization profiles in the real world setting

Tislelizumab

General:
• Tislelizumab studies in the indications included in the Clinical Development Plan: Lung cancer, Esophageal cancer, Gastric cancer, Hepatocellular cancer, Nasopharyngeal cancer addressing specific populations not included in pivotal clinical trials
• Early stages in the above mentioned indications (neo/adjuvant)
• RWE in US/Europe/Japan populations in the above mentioned indications, data on patients characteristics, treatment patterns.

Biomarkers:
• Predicting biomarkers of response to anti-PD1 and primary/acquired resistance to anti-PD1
• Innovative combinations with Tislelizumab and new MoA molecules for patients’ unmet needs in prioritized areas (NSCLC, CRC, PDAC, GC and TNBC)

Out of scope:
• Indications not mentioned as priority above
• Tislelizumab in combination with established products

Ophthalmology

Brolucizumab (Beovu)

Indication: neovascular Age-related Macular Degeneration (nAMD)

• Studies investigating innovative assessment methods:
  ◦ functional efficacy end-points, e.g. reading speed, contrast sensitivity and anatomic end-points
  ◦ new imaging tools e.g. angio-OCT and Ultrawide Field imaging
• Studies aimed to evaluate effectiveness with real-world usage of brolucizumab, including treatment patterns, anatomical outcomes, treatment burden and adherence/compliance, impact on QoL
• Studies investigating brolucizumab in sub-populations, e.g. CNV sub-types or PED and other VEGF-driven retinal diseases, e.g. Mac-Tel; Central Serous Chorioretinopathy and similar
• Studies investigating biomarkers or genetic markers which could better predict outcomes to improve
patient care

- Studies utilizing novel PRO endpoints and evaluations
- Studies involving use of digital technology for improved patient compliance (e.g. home monitoring devices)

Out of scope:
- Mechanistic/Pre-clinical studies aimed to evaluate and understand PK/PD or systemic VEGF levels and similar
- H2H studies with insufficient statistical power (i.e. small sample size) evaluating BCVA as primary efficacy objective
- Studies (single arm or H2H) targeting safety as primary or key secondary end-point
- H2H studies versus unlicensed bevacizumab or licensed biosimilars of any anti-VEGF agents

**Lifitegrast (Xiidra®) and ECF843**

Indication: dry eye disease

- Characterization of DED and/or DED patient types (i.e. Sjogren’s Disease, Meibomian Gland Dysfunction)
- Tools to measure DED signs and symptoms objectively
- Simplifying diagnosis and measuring disease progression (i.e. biomarkers)
- Functional vision testing
- Effect of screen use on OSD
- Tools to improve patient compliance

Out of scope:
- Studies comparing to artificial tears
- Head to head studies

**SAF312**

Indication: Chronic Ocular Surface Pain (COSP)

- Characterization of patient cohorts / disease populations with chronic ocular surface pain
- Understanding the pathophysiology, neurology and inflammatory components of COSP
- Characterization of COSP signs and symptoms
- Tools to measure chronic ocular surface pain signs and symptoms objectively
- Patient-Reported Outcomes for chronic ocular surface pain assessments
- Characterization of the treatment paradigm and referral pathway for ocular pain patients
- Management guidelines for chronic ocular surface pain
- Studies exploring the origin of symptoms on the ocular surface (such as burning, photophobia; etc)
- Studies focused on other potential TRPV1 mediated chronic conditions (such as VKC, AKC; etc)
- Studies exploring biomarkers present in patients reporting COSP
- The social and economic burden of COSP

Out of scope:
- Studies treating the underlying cause of the ocular surface pain
- Studies vs. systemic pain management regimes

**Voretigene neparvovec (Luxturna®)**

Indication: ocular gene

- Epidemiology studies
• Prospective non-interventional studies to follow-up efficacy & safety of treated patients
• Patient-reported outcome projects in IRD patients
• Patient identification, retina viable cells & peer to peer engagement
• Molecular diagnosis & genotyping projects (including variances of uncertain significance)
• Novel surgical procedures for drug delivery
• Novel approaches to reduce time of diagnosis of IRD patients
• Out of scope:
  ○ Studies in non-ocular disease
  ○ Studies in patients below the age of 12 months
  ○ Intervventional studies in which drug is requested

Respiratory & Allergy

Enerzair Breezhaler (QVM149; indacaterol acetate, glycopyrronium bromide and mometasone furoate [IND/GLY/MF])

• Effectiveness of IND/GLY/MF (LABA/LAMA/ICS), incl. benefits on symptom relief
• Benefits of the Breezhaler (Concept 1) and/or digital companion (sensor & app), incl. characterization of the patient population who benefits most from digital tools
• Anti-inflammatory action of GLY (LAMA)

Omalizumab (Xolair)

• Asthma: efficacy/effectiveness/Safety of Xolair on asthma and in comorbid conditions
• Nasal Polyposis (NP): effectiveness of Xolair in NP, effect of Xolair on the NP biology, AFRS Xolair data (Allergic Fungal RhinoSinusitis)
• Food Allergy FA: Xolair effectiveness on FA, data on the role of IgE in all FA, data on the food allergen specific IgE
• Chronic Spontaneous Urticaria: studies on treatment in early CSU disease, Patient reported/patient centric outcomes, effect of Xolair on the CSU biology, RWE & Long-term Safety and efficacy, CINDU, other dosing regimens (eg > 300 mg/4w), digital technology for improved patient compliance; disease modifying capabilities, patients <12 y.o
• Other indications with rationale to support the role of IgE and with high medical need

How do I submit an IIT request?

IIT requests are submitted via the Novartis Grants, External Studies and Managed Access System or GEMS portal. Please submit your concept by clicking here.

Guidance on using the GEMS portal is available here:

Novartis GEMS portal external user guide (PDF 0.1 MB)

10/11
For IIT related questions, please contact the medical team in your Novartis local country office.

Source URL: https://www.novartis.com/healthcare-professionals/investigator-initiated-trials-studies

List of links present in page

- https://www.novartis.com/healthcare-professionals/investigator-initiated-trials-studies
- https://www.novartis.com/about/locations
- https://www.cybergrants.com/pls/cybergrants/quiz.display_question?x_gm_id=2932&x_quiz_id=9952