

Investigator initiated trials / studies (clinical) and investigator initiated research (non-clinical)

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

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

The proposed research must offer meaningful scientific objectives and be supported by a valid study design. Privacy rights, safety and welfare of patients and animals shall be fully secured.

IITs are defined by Novartis as “studies with scientific and medical merit developed and sponsored by an independent investigator or academic sponsor. An IIT may be a clinical study conducted without the participation of Novartis, for which the IIT sponsor requests Novartis to provide either funding, drug product or both.”

IIRs are defined by Novartis as “non-clinical research conducted by an independent third-party sponsor to evaluate the effects, properties or profile of a Novartis drug that is conducted in animals or in vitro assays or utilizes previously collected human tissue*.”

Note: Any monetary request intended for a specified purpose to support medical/scientific research, education, policy initiatives, and patient advocacy activities, where Novartis will receive no benefit, should not be considered as IIT or IIR as these are classified as Grants. [Read more information for Grants.](#)

[Novartis position on Investigator Initiated Trials \(IITs\) and Investigator Initiated Research \(IIRs\) \(112 KB\)](#) [IIT guidance for investigators \(719 KB\)](#)

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.

To broaden [Novartis' Commitment to Patients and Caregivers](#) and encourage the wider research ecosystem to improve outcomes for patients and change the practice of medicine, we encourage qualified investigators to inform their research proposals and project executions with the insights of and engagement with people living with conditions of study. We encourage the research community to actively seek out and listen to insights from the patient community to inform design, operations, and meaningful use of participant data for and with patients. Consider the available resources from TransCelerate's [Patient Experience](#) Initiative, the [IMI PARADIGM Patient Engagement Toolbox](#), and [PFMD.org](#) to enhance your project.

- [Global – Strategic areas of interest](#)
- [US – Strategic areas of interest](#)

Cardiovascular, Renal & Metabolism

Atrasentan (Vanrafia)

Without drug (IgAN, FSGS)

- Diagnosis and classification

- Additional ways to foster (earlier) diagnosis of IgAN and FSGS beyond biopsy
- Validate outcome measures (endpoint validation, validation of definitions) including patient-related outcomes, treatment targets, partial remission / remission and relapse criteria in IgAN
- Pathophysiology and biomarkers
 - Studies evaluating the mechanism of anemia with ERAs
 - Role of the endothelin system in IgAN and FSGS
 - Identification of approaches that lead to better characterization, management and/or correlation with outcomes in IgAN and FSGS (eg identification of biomarkers, genetic analysis, novel imaging technologies or biopsy-based studies)
- Disease burden
 - Burden of disease (clinical, economic and/or humanistic burden) in IgAN and FSGS
 - Epidemiological studies - Prevalence, treatment patterns and RWE, sex/geography differences (incl. registries) in IgAN and FSGS
 - Disease characteristics and clinical outcomes in patients with skin of color, ethnic minorities, or populations so far underrepresented in clinical trials. Gender related differences.
 - Gaps in the optimal care and management of IgAN and FSGS patients
 - Impact of patient education programs
 - Patient perspective on disease, treatment options and QoL

With Drug (IgAN, FSGS)

- Mode of action
 - Mechanistic studies using atrasentan in IgAN and FSGS, including biomarkers, biopsy and novel imaging techniques (eg MRI)
 - Studies evaluating non-hemodynamic effects of atrasentan in IgAN and FSGS
 - Studies evaluating the effects of atrasentan on pain in IgAN and FSGS
- Treatment optimization
 - Studies evaluating novel implementation protocols in IgAN
 - Evaluation/biomarker analysis of subgroups of patients included in the overall study populations in the atrasentan CDP
 - Studies in IgAN utilising SGLT2i and/or other novel treatments in combination with atrasentan
 - Studies evaluating the early use of atrasentan initiated alongside RASi +/- SGLT2i, or atrasentan alone in IgAN
- Expanded populations in IgAN
 - Studies of atrasentan in patients with IgAN with less severe disease (eg. lower proteinuria >0.5g/d)
 - Studies of atrasentan in patients with IgAN in more severe disease (eg. CKD stage 4, eGFR 15-29 ml/min/1.73m²)
 - Studies evaluating atrasentan in transplant patients with recurrent IgAN

Out of scope (IgAN, FSGS)

- Pediatric studies in IgAN and FSGS
- Studies exploring different dosing regimens to those currently being evaluated in current clinical development programs for IgAN and FSGS
- Head-to-head comparisons with other treatments for IgAN and FSGS
- Studies in IgAN including patients with eGFR <15 ml/min/1.73m² (CKD stage 5)

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)
- Early post-event implementation
- Stroke
- PAD

- LDL-C lowering in under-represented population
- Adherence vs. other LLTs

Mechanistic Studies in secondary prevention

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

Mechanistic Studies in primary prevention and/or patients with statin-intolerance

- Remodeling, fibrosis, inflammation
- Plaque burden regression/modification
- Assessment techniques (IVUS, echo, CCTA, OTC, MRI) 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 adults with HoFH and/or different populations than ASCVD and ASCVD equivalent
- CVOT trials
- Head-to-head efficacy/safety studies with other lipid lowering therapies
- Pre-Clinical Proposals (separate process)

Studies involving drug for any indication(s) currently in clinical development and not yet approved

Iptacopan (Fabhalta)

Without drug (IgAN, C3G, IC-MPGN, aHUS, LN, AAV, FSGS)

- Additional ways to foster (earlier) diagnosis of glomerulopathies, beyond biopsy including diagnostic research leveraging use of artificial intelligence in IgAN, C3G, IC-MPGN, aHUS, LN, AAV, FSGS
- Identification of approaches that lead to better characterization, management and/or correlation with outcomes in IgAN, C3G, IC-MPGN, aHUS, LN, AAV, FSGS eg identification of biomarkers, genetic analysis or biopsy-based studies
- Burden of disease (clinical, economic and/or humanistic burden in IgAN, C3G, IC-MPGN, aHUS, LN, AAV)
- Epidemiological studies - Prevalence, treatment patterns and RWE, sex/geography differences (incl. registries) in IgAN, C3G, IC-MPGN, aHUS, LN, AAV
- Role of the complement system in complement mediated kidney diseases

With drug (IgAN, C3G, IC-MPGN)

- Mechanistic studies using iptacopan including biopsy in IgAN C3G, IC-MPGN
- Subgroups of patients that are included in the overall study population in indications in the iptacopan development program in IgAN, C3G, IC-MPGN
- Studies evaluating iptacopan in transplant patients with recurrent IgAN or recurrent C3G
- Evaluation/biomarker analysis for subgroups of patients included in the overall study population in IgAN, C3G and IC-MPGN
- Preclinical research studies in IgAN, C3G and IC-MPGN
- Studies investigating combination, sequencing of targeted treatments in IgAN

Out of scope (with drug)

- Pediatric studies
- Studies exploring different dosing regimens to those currently being evaluated in current clinical development program
- Head-to-head comparisons
- Studies including patients with GFR <20 ml/min/1.73m²

Without drug

- Epidemiology associated with elevated Lp(a)
 - Patient characterization, identification, and genetic risk across sub-groups
 - Plaque characteristics and differences across patient sub-groups
 - Association & impact on different types of CVD (ischemic stroke, CAVS, PAD), various vascular beds, and other diseases (e.g., AF, kidney disease, diabetes)
- Distinct and unique pathophysiology of Lp(a)
 - Insights on the pro-inflammatory or pro-thrombotic mechanisms impacted by Lp(a)
 - Unique features of Lp(a)
- Quantification of Lp(a) role in CV risk assessment tools
 - Quantification of Lp(a) contribution to global CV risk and in light of other CV risk factors
 - Risk score calculators incorporating Lp(a)
 - Patient perception on contribution of Lp(a) to CVD and CV risk
- Lp(a) testing and global CV risk management
 - Implementation of Lp(a) testing in CVD management pathways
 - Clinical and economic value of Lp(a) testing
 - Guidance on management of currently modifiable risk factors in the setting of elevated Lp(a)

Out of scope:

- Comparison / association with LDL-C
- Studies involving drug for any indication(s) currently in clinical development and not yet approved

Sacubitril/Valsartan (Entresto)

Heart Failure

- RWE or Implementation Science studies on improvements of HF care through increase in GDMT
- RWE studies with sac/val in Chronic Heart Failure with reduced EF
- RWE studies with sac/val in Chronic Heart Failure with mildly-reduced or preserved EF - in geographies where it is in-label
- RWE studies with sac/val in HTN - in geographies where it is in-label

Out of scope:

- Comparative effectiveness studies vs other MoA, e.g. SGLT2i, MRA, BB
- Studies in non-cardiovascular disease
- Studies in patients with valvular disorders not related to HF
- Studies in children (<18 years)
- Mechanistic Studies in HF including but not limited to those looking at:
 - Remodeling, fibrosis, inflammation
 - Cardiac function (including diastolic function)
 - Cardiac biomarkers
- Studies in populations with specific, less well studied / documented HF etiologies, e.g. chemotherapy /toxicity induced HF

Zigakibart

Without drug

- Diagnosis and classification
 - Additional ways to foster (earlier) diagnosis of glomerulopathies (IgAN), beyond biopsy and its impact on outcomes
 - Validate outcome measures (endpoint validation, validation of definitions) including patient-related outcomes, treatment targets, partial remission / remission and relapse criteria in IgAN
- Pathophysiology and biomarkers
 - Role of APRIL system in IgAN, Gd-IgA1 and autoantibodies in IgAN Including the subtype characterisation

- Identification of approaches that lead to better characterization, management and/or correlation with outcomes in IgAN eg identification of biomarkers, genetic analysis or biopsy-based studies
- Predictive models for APRIL inhibition responses across disease phenotypes/genotypes
- Disease burden
 - Burden of disease (clinical, economic and/or humanistic burden) in IgAN
 - Epidemiological studies - Prevalence, treatment patterns and RWE, sex/geography differences (incl. registries) in IgAN Disease characteristics and clinical outcomes in patients with different racial and ethnic backgrounds, or populations so far underrepresented in clinical trials. Gender related differences.
 - Gaps in the optimal care and management of IgAN patients
 - Impact of patient education programs
 - Patient perspective on disease, treatment options and QoL

With drug

- Pre-clinical
 - Mechanistic studies in IgAN
 - Preclinical evaluations aimed at demonstrating MoA-pathway activity and/or differentiation
- Clinical
 - Evaluation of zigakibart in expanded IgAN patient populations: Transplant patients with recurrent IgAN, IgA vasculitis with nephritis patients, Low proteinuria (0.5 – 1 g/d)

Out of scope

- Pediatric studies
- Studies exploring different dosing regimens to those currently being evaluated in current clinical development program
- Head-to-head comparisons
- The value of tight control (treat to target) versus conventional management strategies in IgAN
- Risk prediction models for which patients may develop hypogammaglobulinemia
- Preclinical (comparison) studies (vs APRIL/BAFF or APRIL/BLyS) and other IgAN treatments
- Duration and withdrawal of therapy / impact of treatment breaks
- Combination evidence
- Studies including patients with GFR <20 ml/min/1.73m²
- Vaccine titer studies or b-cell subset characterization

Gene Therapies

Zolgensma IV

Areas of interest by product

- Demonstrating or validating care needs for SMA populations post OAV101 Treatment-safety related items
- Expansion of treatment with OAV101 to patient populations not included in clinical trials (e.g. older/heavier, 4 copies, switch therapy, ambulatory)
- Value of OAV101: Cost of care, Quality of life, and Caregiver Burden-Cost effectiveness
- Methods/Processes to assess the efficacy and durability of OAV101 (e.g. bulbar function)
- Biomarkers for efficacy

Out of scope

- Clinical Trials involving OAV 101 re-dosing
- Study of OAV101 alternative doses/maximum dose
- Head-to-head comparison with other therapies and combination with other MDT
- Basic Science research that request use of OAV101

OAV101 IT

Areas of interest by product

- Interventional Studies of OAV IT in patients not included in clinical trials (e.g. ambulant SMA patients, severe scoliosis)
- Non-interventional Studies of OAV IT assessing sleep, bulbar function, scoliosis and respiratory function, head steadiness and independence.
- Studies on biomarkers assessing clinical response to OAV IT

Out of scope

- Clinical Trials involving OAV 101 re-dosing
- Study of OAV101 alternative doses/maximum dose
- Head-to-head comparison with other therapies and combination with other MDT
- Basic Science research that request use of OAV101

Global Health

Adakveo, Ryverna (Crizanlizumab, SEG101)

- Studies with crizanlizumab in sickle cell disease and related complications
 - e.g- renal, leg ulcer, stroke, AVN, adolescents with SCD, priapism, splenic sequestration, VOCs
- Mechanistic studies with crizanlizumab
- Predictors of response to crizanlizumab
- SCD biomarkers

Out of scope:

- IIT requests from countries outside of US, SSA, Brazil
- IIT requests in non-SCD indications

HU, HU-FCT (Hydroxyurea)

- Studies with HU in sickle cell disease and organ protection
 - e.g., spleen, lungs, kidneys
- Societal and economic impact of HU/HU-FCT on LMIC
- Studies with HU-FCT looking at treatment/stroke prevention in LMIC
- HU-FCT preference by caregivers

Out of scope

- IIT requests from countries outside of SSA, Brazil and India
- IIT requests in non-SCD indications

Ganaplacide/Lumefantrine

- Efficacy against non-falciparum malaria (blood stage)
- Efficacy when used in combination with tafenoquine
- Special patient groups- >65 years, >70kg
- Population impact of use in low transmission settings

Out of scope

- Use in traveler prophylaxis

Special populations

- Treatment of malaria in pregnancy
- Treatment Studies in malnourished individuals

Immunology

Ianalumab

Clinical data, outcomes & RWE (with drug):

- RWE studies on clinical effectiveness and safety of lanalumab in Sjogren's disease
- Clinical outcomes with lanalumab across different manifestations and domains of Sjogren's disease
- Clinical outcomes with lanalumab by gender, race, skin of colors, ethnic minorities and access to health care systems in Sjogren's disease
- Research investigating clinical impact of lanalumab on Sjogren's disease systemic and organ complications such as neuropathies, interstitial lung disease, renal disease, vasculitis, etc

Implementation Science/HCS research.

- Population-based epidemiology studies investigating the impact of autoimmune diseases on patients; comorbidities; studies of real-world treatment patterns (incl. e.g. overuse of corticosteroids, delays in systemic therapies initiation or Treat-to-Target approaches, etc)
- Identification of predictors and patient characteristics associated with rapid progression or poor outcomes in autoimmune diseases
- Impact of early intervention and treat-to-target on patient outcomes
- Impact of systemic treatments on prevention or reduction of long-term comorbidities and complications in autoimmune diseases
- Research utilizing novel imaging modalities for early diagnosis and monitoring of treatment responses
- Research evaluating treatment impact on patients' symptoms, such as fatigue, pain, mental and sexual well-being,
- Research evaluating patients perspectives in terms of unmet needs, current treatment satisfaction, disease management, treatment adherence, PROs, and shared decision-making across autoimmune diseases

Exploratory/ mechanistic studies:

- Role of B-cells in the pathogenesis of Sjogren's disease, Systemic Lupus Erythematosus, Systemic Sclerosis, and other autoimmune conditions
- Impact of BAFF blockade on Sjogren's disease, Systemic Lupus Erythematosus, Systemic Sclerosis, and other autoimmune conditions
- Identification and validation of new clinical outcome measures or PROs for clinical trials or real-world practice use across autoimmune diseases
- Studies employing digital or AI based technologies e.g. in silico models, machine learning techniques, telemedicine etc. to support clinical diagnosis, clinical management, prediction of disease trajectories, prediction of treatment responses

Out of scope

- Studies on safety topics e.g. infections (tuberculosis, HIV, viral hepatitis), high-risk patients
- Studies with combination biologics
- Clinical comparative studies with other treatments
- Pediatric studies
- Studies exploring different dosing regimens as currently investigated

Secukinumab (Cosentyx)

Indications: axSpA (axial spondyloarthritis), incl. r-axSpA (radiographic) and nr-axSpA (non-radiographic)

Clinical data, outcomes & RWE:

- Long term RWE studies on clinical efficacy, structural progression & safety of secukinumab
- Clinical outcomes with Secukinumab across different manifestations of axSpA , by gender and race

Implementation Science/HCS research.

- Identification of Predictors of structural progression and treatment algorithm related to structural progression
- Impact of early intervention and treat-to-target on patient outcomes
- impact of Secukinumab on prevention or reduction of Comorbidities
- Research Use of Novel imaging modalities for early diagnosis, pathogenesis of disease and monitoring of

Secukinumab response

- Evaluate the impact of Secukinumab and treatment strategy to reduce Fatigue and pain

Exploratory/ mechanistic studies:

- New classification criteria of AxSpA and differences in pathogenesis of axSpA vs. axial PsA.
- Role of IL-17A in the pathogenesis of axial, peripheral manifestations and comorbidities of AxSpA
- Role of IL-17A across the spectrum of spondyloarthritides (SpA)

Out of scope

- Studies on safety topics e.g. infections (tuberculosis, HIV, viral hepatitis), high-risk patients
 - Studies with combination biologics
 - Clinical comparative studies with other treatments
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Indications: Psoriatic arthritis (PsA)

Clinical data, outcomes and RWE:

- Long term RWE studies on clinical efficacy, inhibition of structural progression & safety of secukinumab
- Long term RWE studies on efficacy, safety and treatment strategy in juvenile PsA (JPsA) and enthesitis-related arthritis (ERA)
- Clinical outcomes with Secukinumab in key manifestations of PsA, by gender, race, ethnic minorities and access to health care systems
- Clinical outcomes with Secukinumab in specific phenotypes (Axial PsA, skin predominant , nail/dactylitis, Oligoarticular predominant)

Implementation Science/HCS research: cost-effectiveness, resource utilization and guideline implementation

- Impact of early treatment and treat-to-target on patient outcomes and resource utilization
- Impact of Secukinumab on prevention or reduction of Comorbidities (e.g.CV, metabolic)
- Research studies on Novel imaging for early diagnosis and monitoring of Secukinumab response
- Evaluate the impact of Secukinumab to reduce Fatigue and pain

Exploratory/ mechanistic studies:

- Role of IL-17A in the pathogenesis of Axial PsA and differences with pathogenesis of. axial PsA vs axSpA
- Roles of different cytokine pathways in the key manifestations of PsA notably axial disease, enthesitis, nail-dactylitis

Out of scope

- Studies on safety topics e.g. infections (tuberculosis, HIV, viral hepatitis), high-risk patients
 - Studies with combination of other biologics
 - Comparative studies with other treatments
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Indications: Psoriasis (PsO)

Clinical data, outcomes and RWE:

- Long term RWE studies on clinical efficacy, & safety of secukinumab, risk factors and prevention of the transition period of PsO to PsA
- Clinical outcomes with Secukinumab by gender, race, skin of colors, ethnic minorities and access to health care systems
- Long term RWE studies on efficacy, safety and treatment strategy in pediatric PsO

Implementation Science/HCS research:

- Impact of early intervention strategy on disease modification in PSO and resource utilization

- Research program designed for early diagnosis and characterization of PSO patients at risk of PsA : disease burden, risk factors, screening tools/app, novel imaging

Exploratory/ mechanistic studies:

- role of IL-17A in the pathogenesis of the transition period PsO to PsA
- Mechanistic study of Secukinumab in Early PsO

Out of scope

- Studies on safety topics e.g. infections (tuberculosis, HIV, viral hepatitis), high-risk patients
- Studies with combination other biologics
- Comparative studies with other treatments

Indications: Hidradenitis Suppurativa

Clinical data, outcomes and RWE:

- Early intervention with Sec and impact on disease progression (including imaging techniques, such as ultrasound)
- Clinical outcomes in subpopulations (e.g. disease phenotypes, Black / African American, super-responders,...)
- Integrating surgical procedures with the administration of Secukinumab for the treatment of HS. (effectiveness and Safety)
- HS comorbidities (Mental Health, Obesity, CV)
- Effects of lifestyle intervention on HS treatment with Secukinumab

Implementation Science / HCS research:

- Quality of care, cost-effectiveness, resource utilization, and guidelines implementation
- AI/ML algorithms and big data approach to improve diagnosis and treatment of HS
- Development and validation of scoring tools / PROs

Exploratory/ mechanistic studies:

- Translational research on pathophysiology - role of IL-17A and other pathways in HS over the course of the disease , and in specific aspects of the disease e.g., Fistula/tunnel development"
- Biomarkers to predict disease and treatment outcomes

Out of scope

- Comparative studies with other treatments
- Combination studies for Secukinumab with other biologic agents
- IV dosing for HS

Indications: GCA and PMR

Pathophysiology and biomarkers

- Biomarkers to monitor subclinical disease activity, predict prognosis and treatment outcomes
- Effects of a mechanism-based approach to therapy
- Pathways involved in refractory/flare GCA
- Biomarkers to predict drug/GC toxicity

Diagnosis and classification

- Standardization of clinical trial endpoints
- Validation of the definition of remission, response, relapse and disease subtypes of importance
- Use of the different imaging techniques for vascular activity, damage assessment and follow-up

Treatment and treatment outcomes

- Implementation Science/HCS research: quality of care, cost-effectiveness, resource utilization, and guidelines implementation
- Disease characteristics and clinical outcomes in patients with skin of color, ethnic minorities, or populations so far underrepresented in clinical trials. Gender related differences
- Assessment of prognosis by demographic, clinical and histological data
- Predictors of response, remission or relapse
- Validation of patient-reported outcomes
- Predictive models for IL-17A inhibition responses across disease phenotypes
- Effect of secukinumab on the development of future vascular complications

Out of scope

- Effect of secukinumab on the development of future vascular complications
- Combination studies of secukinumab with other biologic agents
- The role of ultrasound for guiding temporal artery biopsy, specific treatment of organ complications

Remibrutinib in Chronic Urticaria

Disease: Chronic Urticaria – CSU (chronic spontaneous urticaria) and CIndU (chronic inducible urticaria)

Product: Remibrutinib

Areas of interest by product

Disease Related Research:

- Population-based epidemiology studies; studies investigating the impact of urticaria on patients; comorbidities; studies of real-world treatment patterns (incl. e.g. overuse of corticosteroids, impact on sleep)
- Studies investigating biomarkers in urticaria aiming to understanding acute -> chronic urticaria transition, biomarkers, identify patients at risk biomarkers predictive of treatment response, biomarkers correlating with the time-course of CSU, biomarkers of permanent remission or relapse, including with remibrutinib
- Studies employing digital technology e.g. in silico models, AI / machine learning techniques, telemedicine etc. to predict disease trajectories, treatment response, disease modification, etc.
- Studies investigating innovative tools to support urticaria management, e.g. digital applications, sleep related
- Long-term CSU observational registries and secondary use of data
- Studies investigating patient preference

Clinical Studies:

- Studies with remibrutinib in CIndU
- Studies with remibrutinib focusing on angioedema
- Remi in acute or early chronic urticaria / prevention of chronic urticaria
- Remi in other potentially BTK mediated conditions like allergic conditions, urticaria vasculitis etc.

Mechanistic Studies:

- Clinical Mechanistic studies assessing the effects of remibrutinib on mast cells, basophils, B-cells
- Clinical mechanistic studies investigating the disease modifying potential of remibrutinib
- Clinical mechanistic studies investigating the impact of remibrutinib on peripheral neuroinflammation

Out of scope

- Head-to-head comparisons of remibrutinib with other active treatments
- Studies investigating remibrutinib in combination with biologics
- Alternative dosing regimens to 25 mg bid developed in the CSU phase 3 clinical program

Neuroscience

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

- Focus on prognosis and diligent monitoring of patients with MS (including data and digital)

- Focus on prognosis and urgent 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

Remibrutinib in RMS

Disease: Relapsing Multiple Sclerosis

Product: Remibrutinib

Areas of interest by product

Follow the science

- Impact on the immune system in and outside the CNS – clinical and preclinical studies
- Direct CNS effects (i.e. microglial activity, synaptogenesis, neuronal function) and correlation with clinical outcomes beyond relapses (e.g. PIRA, disability improvement) and with some patient outcomes (e.g. cognition, fatigue)
- Impact on chronic inflammation and correlation with linked clinical and paraclinical outcomes (disability measures, such as EDSS/MSFC, imaging measures, fluid biomarkers)

Safety related:

- BTKi – vaccine response
- Pregnancy registries (in line with initiatives already in place)

Identify unmet need under current DMTs:

- Patient preference
- Tolerability and safety concerns (what, when, to whom – patient profile-)
- Effectiveness gaps under HET (what, when, to whom – patient profile -)

Out of scope

Progressive phenotypes of MS (naSPMS or PPMS); hepatotoxicity

Oncology

Asciminib (Scemblix)

Studies in adult patients Chronic Myeloid Leukemia in Chronic Phase (CML-CP) in early treatment lines, investigating:

- Long-term safety and tolerability
- Clinical efficacy and safety in real-world setting
- Treatment sequencing
- Patient-reported outcomes (PROs) and Quality of Life (QoL)
- Patients with CML-CP and additional T315I mutation
- Response to asciminib in patients with pre-existing mutations other than T315I or treatment approaches in patients with emerging mutations under asciminib, including compound mutations

Studies exploring additional high-need patient populations other than CML-CP:

- Patients with Philadelphia-positive acute lymphoblastic leukemia (Ph+ALL)
- Exploratory high risk CML populations such as patients with additional genomic alterations
- CML-AP/BC

Studies in Treatment Free Remission (TFR)

- Studies aiming to improve deep molecular responses, increase the eligibility for TFR attempts or reduce the risk of

relapse after treatment discontinuation

- Combination approaches of asciminib with non-ATP-TKI compounds

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

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) – PNH and CAD;
5. Epidemiology studies (incl. registries) – 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

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

[177Lu] Lu-DOTA-TATE (Lutathera®)

- Studies (other than prospective design) describing optimal timing and sequence of treatment in advanced or metastatic GEP-NET patients
- Studies of Lutathera in advanced or metastatic NET patients in combination with other anti-cancer treatments, including chemotherapy (also bolus 1L), immuno-oncology therapies, tyrosine kinase inhibitors (TKIs), PARP-inhibitors, CDK4/6 inhibitors, or other upcoming treatments (if supported by MoA rationale)
- Retrospective studies describing long-term safety or health economic aspects
- Studies on biomarkers to predict and prognosticate treatment in GEP-NET

[177Lu] Lu-PSMA-617 (Pluvicto®)

Indication: Prostate Cancer

- mHSPC, mCRPC - **Sequencing RWE** - Sequential use of different radioligand therapies (alpha- or beta-emitter); Treatment optimization
- HRLPC, BCR - **Combinations** - Efficacy and safety of 177Lu-PSMA-617 combinations to overcome resistance and to improve efficacy outcomes
- mHSPC (OMPC) - **Low volume disease** - Efficacy and safety of 177Lu-PSMA-617 in low volume disease
- mHSPC - **Alternative dosing** - Adaptive and alternative treatment regimens with 177Lu-PSMA-617 monotherapy or in

combinations

- HRLPC, BCR - **Treatment effect in earlier stages** - Efficacy and safety of radioligand therapy (alpha- or beta-emitter)
- mHSPC, mCRPC - **Subpopulations** - Impact of 177Lu-PSMA-617 efficacy and safety in patient populations with sub-optimal outcomes, including patients distinct mutations (e.g., PTEN-loss, AKT, DDR), patients CNS mets, liver mets etc.
- All disease stages - **Long-term safety** - Retrospective analysis to predict long-term safety events
- HRLPC, mHSPC, 1L-2L mCRPC - **Translational Research** - Treatment effect on disease biology
- HRLPC, mHSPC, 1L-2L mCRPC - **Imaging** - Understanding PSMA expression in different stages of prostate cancer

Beyond GU

- Brain Metastasis (secondary malignancies)
- Ovarian Ca
- NSCLC
- GBM: microenvironment and translational research (MoA deeper understanding)
- GBM: Other mode of administration in GBM (not IV)
- Hepatocellular Carcinoma
- High grade gliomas
- Others PSMA-expressing/PET-avid tumors
- Imaging studies
- Pediatric indications

Ribociclib (Kisqali)

- HR+/HER2- studies in breast cancer
 - Exploring data on CDK4/6 inhibitor rechallenge
 - Exploring ribociclib with novel/emergent compounds
 - Utilizing real world data and/or digital health technologies
 - Utilizing patient reported outcomes (PRO)

Out of scope:

- Any area outside HR+/HER2- breast cancer
- Any study in overlap with ongoing Novartis-sponsored/supported studies

Oncology

Ribociclib (Kisqali)

HR+/HER2- Studies in Breast Cancer

- Exploring ribociclib with novel/emergent compounds
- Utilizing real-world data (RWD) and/or digital health technologies
- Enhances the treatment experience of patients

Out of Scope

- Any area outside HR+/HER2- breast cancer
- Any study in overlap with ongoing Novartis-sponsored/supported studies

177LuDOTATATE (Lutathera) / 68GaDOTATATE (Netspot)

GEP & Bronchopulmonary NET

- Re-treatment/Re-challenge with 177LuDOTATATE (after initial 4 cycles)
- Combinations of 177LuDOTATATE with other agents with potential to improve efficacy
- Sequencing studies with 177LuDOTATATE
- Long-term safety of 177LuDOTATATE
- Efficacy/Safety of 177LuDOTATATE in specific patient subgroups

Other SSTR+ Tumors

- Role of ¹⁷⁷LuDOTATATE in the management of patients with other SSTR+ tumors

NETSPOT for Imaging

- Role of ⁶⁸GaDOTATATE in GEP-NET and other SSTR2+ tumors

[⁶⁸Ga]Ga-FAP-2286

Imaging Studies in FAP-expressing Solid Tumors

- Role of FAP PET in diagnosis, staging, clinical decision-making, and treatment response
- Studies exploring FAP PET as an imaging biomarker: correlation with other biomarkers such as histological, molecular, and genetic subtypes
- Understanding FAP expression in benign/inflammatory processes in relation to FAP RLT safety and patient selection for therapy

²²⁵Ac-PSMA-617

- Use of PSMA-targeted PET imaging agents in prostate cancer (e.g., patient selection, treatment assessment)
- Treatments up-regulating PSMA expression in prostate cancer
- Methods of preventing and/or managing RLT-induced xerostomia
- Real-world evidence in prostate cancer for ²²⁵Ac-PSMA-617
- Health disparities in advanced prostate cancer
- Sequencing with ¹⁷⁷Lu-PSMA-617

Tisagenlecleucel (Kymriah)

- Essential factors for selecting patients for tisagenlecleucel therapy to improve safety and/or response for their approved indications
- Essential factors for sequencing tisagenlecleucel therapy with other therapies and determining outcomes for their approved indications
- Novel combinations of therapies with tisagenlecleucel to improve response and/or safety for their approved indications
- Study outcomes of tisagenlecleucel administered at various sites (e.g., inpatient, outpatient, community hospital, community practice) for their approved indications

Asciminib (Scemblix)

CML-CP in Earlier Lines (1L & 2L)

- Sequencing of TKIs, clinical efficacy, and safety in real-world settings
- Patient-reported outcomes (PROs) and quality-of-life issues with current CML therapies
- Biomarkers for TFR and safety
- Long-term safety and tolerability
- Studies aiming to improve eligibility to attempt TFR and reduce the risk of relapse after TFR attempts
- Response to asciminib in patients with pre-existing BCR::ABL1 mutations other than T315I, or treatment approaches in patients with emerging mutations under asciminib, including compound mutations

CML-BC and Ph+ ALL

- Efficacy and safety of asciminib in selected ALL settings (Ph+, Ph-like)
- Exploratory high-risk advanced phase CML populations such as patients with additional genomic alterations
- TKI-based combinations addressing high unmet need populations (CML-AP/BC)

Out of Scope

- Use in non-BCR::ABL diseases

[177Lu]Lu-PSMA-617 (Pluvicto)

- Investigating retreatment or extended treatment with ¹⁷⁷Lu-PSMA-617
- Real-world evidence in prostate cancer for ¹⁷⁷Lu-PSMA-617
- Health disparities in advanced prostate cancer

CRM

Inclisiran (Leqvio)

- ASCVD MOA – atherosclerotic plaque composition changes
- Differentiation of inclisiran vs. other LLTs in a real-world setting (e.g., adherence, implementation)

Pelacarsen / Lp(a)

Non-drug IITs

Epidemiology associated with elevated Lp(a)

- Patient characterization, identification, and genetic risk across sub-groups.
- Association and impact on different types of CVD (e.g., ischemic stroke, PAD), polyvascular disease, and other cardiovascular-related diseases.

Distinct and unique pathophysiology of Lp(a)-related CVD

- Insights on the pro-thrombotic mechanisms impacted by Lp(a).
- Unique features of Lp(a).

Patient perception of the contribution of Lp(a) to CVD and cardiovascular risk

Lp(a) testing and global cardiovascular risk management

- Implementation of Lp(a) testing in CVD risk evaluation.
- Clinical and economic value of Lp(a) testing.

Out of Scope:

- Relationships between Lp(a) and LDL-C, and their independent contributions to Lp(a)-related disease risk.
- Lp(a) in non-cardiovascular-related disease.

Atrasentan (Vanrafia)

Disease-related

- Studies evaluating the role of the endothelin pathway in proteinuric kidney diseases
- Patient journey mapping and practice change initiatives to improve outcomes and reduce disparities

Drug-related

- Studies on combination strategies with atrasentan in IgAN

Out of Scope:

- Pediatric studies involving drug treatment
- Studies exploring dosing regimens other than the currently FDA-approved dose
- Head-to-head comparisons with other approved products
- Studies including patients with eGFR <15 mL/min/1.73 m² (CKD stage 5)

Iptacopan (Fabhalta)

Disease-related

Studies evaluating the role of the complement system in complement-mediated kidney diseases.

Patient journey mapping and practice change initiatives to improve outcomes and reduce disparities.

Drug-related

Studies on combination strategies with iptacopan in IgAN.

Out of Scope

Pediatric studies (with drug).

Head-to-head comparisons with other approved products.

Studies including patients with GFR <20 mL/min/1.73 m².

Iptacopan PNH (Fabhalta)

With drug

- Mechanistic studies in PNH.
- Studies evaluating factors associated with or predictive of treatment outcome in PNH.

Without drug

- Approaches to facilitating and expediting diagnosis
- Identification of biomarkers that lead to better characterization, management, or correlation with outcomes.

Out of Scope

- Pediatric studies.
- Clinical trials exploring different dosing regimens than those currently being evaluated in the clinical development program.
- Clinical trials combining iptacopan with immunosuppressant and anti-C5 treatments.
- Head-to-head comparisons.
- Studies in other non-complement-mediated hematologic diseases.

Neuroscience

Ofatumumab (Kesimpta)

Multiple Sclerosis

- The experience of use of OMB in sub-populations of RMS (e.g., African American and Hispanic patients, and different age groups).
- The impact of OMB on MS comorbidities and patient-centric outcomes.
- The therapeutic role of OMB in MS, including efficacy, safety, tolerability, and use in treatment-naïve patients.
- The impact of OMB on both fluid and digital biomarkers in MS.
- The pathophysiology of MS (including the mechanism of action of OMB and its effects on MS pathophysiology) and the burden of disease associated with MS (including the impact of OMB).
- Innovative neuroimaging techniques used to measure biomarkers of MS disease, MS inflammation, axonal integrity, and function (including the effects of OMB).
- The long-term impact of B-cell therapy on the immune system.
- Impact of B-cell-depleting therapies on immune system function over time, with particular focus on the non-B-cell compartment.

Remibrutinib

Multiple Sclerosis

- The role for remibrutinib in sequencing of treatments.

- The impact on imaging, including SELs, PRLs, and cortical lesions.
- The impact on the biology of progression, including PET imaging, cognition, fatigue, and depression outcomes.
- Effect of remibrutinib on CNS processes, including blood–brain barrier (BBB) transmigration and microglial activation.
- The proteome profiling effects of remibrutinib.

Myasthenia Gravis

- Development of biomarkers and endpoint exploration for clinical trial use.
- Impact on macrophage involvement in disease pathophysiology at the neuromuscular junction (NMJ).
- Effect of remibrutinib on steroid use and its potential to reduce corticosteroid exposure.

Onasemnogene abeparvovec-xioi (Zolgensma)

In Scope

- Holistic and societal impact of Zolgensma IV, including clinical transformation, economic impact, quality of life, and societal effects.
- Biomarkers for efficacy.

Out of Scope

- Clinical trials involving Zolgensma IV re-dosing.
- Studies of alternative Zolgensma IV doses or maximum dose.
- Basic science research requesting the use of Zolgensma IV.
- Methods or processes to assess the efficacy and durability of Zolgensma IV (e.g., bulbar function).

Onasemnogene abeparvovec-brve (Iltvisma)

In Scope

- Long-term, multi-domain functional and clinical outcomes, including sleep, bulbar function, head control, scoliosis, respiratory function, and overall independence.
- Reproductive outcomes, including fertility and reproductive health.
- Biomarker and mechanistic evidence generation, including biomarkers predictive of clinical and functional response and durability.
- Overall survival, caregiver and societal impact, long-term care needs and cost/resource implications, and the impact of delaying treatment.

Out of Scope

- Interventional studies of OAV101 IT in patient types not included in clinical trials.
- Clinical trials involving OAV101 IT re-dosing or OAV101 IT dosing following OAV101 IV.
- Studies of alternative OAV101 IT doses or maximum dose.
- Head-to-head comparisons with other therapies and combination with other MDTs.
- Studies of OAV101 IT in patients under 2 years of age.
- Comparative studies between Zolgensma IV and OAV101 IT.

Immunology

Remibrutinib - Urticaria

Disease Related Research

- Population-based epidemiology studies; studies investigating the impact of urticaria on patients; studies of real-world treatment patterns (including, for example, overuse of corticosteroids and impact on sleep); and studies of patient preference, patient experience, and satisfaction (qualitative).
- Studies employing digital technology, such as in silico models, AI-enabled or machine learning techniques, telemedicine, and related approaches to predict disease trajectories, treatment response, disease modification, and similar outcomes.
- Studies investigating innovative tools to support urticaria management, including digital applications and sleep-related

tools.

- Long-term CSU/CIndU observational studies and secondary use of data.

Clinical Studies

- Studies with remibrutinib in CSU/CIndU.
- Proof-of-concept studies in new allergic and dermatologic indications.

Mechanistic Studies

- Mechanistic studies assessing the effects of remibrutinib on mast cells, basophils, B cells, and other relevant immune pathways in vitro or ex vivo.

Out of Scope

- Head-to-head comparisons of remibrutinib with other active treatments.
- Studies investigating remibrutinib in combination with biologics.
- Alternative dosing regimens to the 25 mg b.i.d. regimen developed in the CSU Phase 3 clinical program.

Ianalumab

- Sjogren's US epidemiology and systemic disease characterization.
- Sjogren's classification, systemic disease recognition, and clinical assessment.
- Sjogren's disease progression: use of ultrasound, clinical assessments, and/or biomarkers.
- Sjogren's symptoms: evidence generation using existing PROs or new SjD symptom assessment modalities.
- Sjogren's disease organ domains: generation of evidence in key disease domains.
- Sjogren's disease and concomitant conditions (e.g., rheumatoid arthritis, lupus) and associated outcomes.
- Sjogren's disease in subpopulations (e.g., African American, Hispanic) and associated outcomes.
- Sjogren's disease burden: clinical, social, economic, and humanistic aspects.
- Biomarker-informed assessment and monitoring in SjD.
- SjD serologic and immunologic characterization and association with clinical outcomes.
- Tissue-level immune activity and disease progression in SjD.

How do I submit an IIT/IIR request?

IIT/IIR 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 \(PDF 0.2 MB\)](#)

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

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List of links present in page

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