

IIT US Areas of Interest

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.

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