NOVARTIS

IIT US Areas of Interest

Oncology

Tisagenlecleucel (Kymriah)

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

Asciminib (Scemblix)

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

- Sequencing of TKIs, clinical efficacy, and safety in real-world setting
- Patient – reported outcomes (PROs) and Quality of life issues with current CML therapies
- TFR and safety biomarkers
- Long-term safety and tolerability
- Studies aiming to improve deep molecular responses, increase the eligibility for TFR attempts or reduce the risk of relapse after treatment discontinuation.
- 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

CML- BC and Ph+ ALL

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

Out of scope

- Use of Non BCR-ABL diseases

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

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

- Use of 177Lu-PSMA-617 in combination with other agents in mHSPC or mCRPC
- Treatments up-regulating PSMA expression in prostate cancer
- Use of 177Lu-PSMA-617 in patients with low or no PSMA expression in mCRPC
- Safety and efficacy of 177Lu-PSMA-617 treatment in solid tumors other than prostate cancer
- Real-world evidence in prostate cancer for 177 Lu-PSMA-617
- Health disparities in advanced prostate cancer
- ADT sparing approaches across Prostate Cancer landscape
- Investigating alternative dosing regimens (cycles, frequency) with 177Lu-PSMA-617 mCRPC
- Sequencing with Actinium

177Lu DOTATATE (Lutathera) / 68Ga DOTATATE (Netspot)

GEP & Bronchopulmonary NET

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

Other SSTR+ Tumors

- Role of Lutathera in the management of patients with other SSTR-positive tumors

NETSPOT for Imaging

- Role of Netspot in GEP-NET and other SSTR2+ tumors

FAP [177Lu] Lu-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/genetic subtype
- Understanding FAP expression in benign/inflammatory processes in relation to FAP RLT safety and patient selection for therapy

Therapeutic Studies

- Role of 177Lu-FAP RLT in FAP-expressing solid tumors
- Use of 177Lu-FAP RLT in combination with standard of care therapies and/or immuno-oncology agents
- Studies investigating the effect of 177Lu-FAP RLT in cancer-associated fibroblasts and tumor microenvironment
- Evaluation of optimal dosing regimens in subpopulations and combinations
- Correlation of RLT efficacy with predictive biomarkers and FAP PET uptake
- Studies exploring alternate routes of administration to improve safety and efficacy

^{225}Ac-PSMA-617 / ^{225}Ac-PSMA-R2

- Investigating alternative dosing regimens (cycles, frequency) with ^{225}Ac-PSMA-617 in mCRPC

- Radioligand therapy in neoadjuvant setting for localized prostate cancer
- Use of ^{225}Ac-PSMA-617 or ^{225}Ac-PSMA-R2 in adjuvant setting in combination with EBRT + ADT +/- abiraterone in patients with localized prostate cancer postprostatectomy with N1M0 on PSMA PET
- Use of ^{225}Ac-PSMA-617 or ^{225}Ac-PSMA-R2 post definitive therapy for localized prostate cancer with biochemical recurrence and PSMA-PET M0 disease
- Use of PSMA-targeted PET imaging agents in prostate cancer (e.g., patient selection, treatment assessment)
- Use of ^{225}Ac-PSMA-617 or ^{225}Ac-PSMA-R2 in combination with other agents in mHSPC or mCRPC
- Treatments up-regulating PSMA expression in prostate cancer
- Use of >6 cycles of ^{225}Ac-PSMA-617 in patients with mHSPC or mCRPC
- Use of ^{225}Ac-PSMA-617 in prostate cancer patients with distinct mutations (e.g., PTEN-loss, AKT, DDR)
- Use of ^{225}Ac-PSMA-617 in patients with low or no PSMA expression in mCRPC
- Real-world evidence in prostate cancer for ^{225}Ac-PSMA-617
- Health disparities in advanced prostate cancer
- Sequencing with 177Lu-PSMA-617

Iptacopan (Fabhalta)

With Drug

- Mechanistic studies in PNH
- Studies evaluating complement factors associated with or predictive of treatment outcome in PNH
- Studies evaluating effectiveness, safety and management of Iptacopan in PNH patients treated in the real-world setting
- Studies evaluating PNH treated patients in the context of bone marrow disorders, such as AA, in the real-world setting
- Studies evaluating the role of factor B inhibition in hematologic complement mediated diseases

Without drug

- Complement mediated diseases in Hematology
- Role of complement system in disease evolution
- Approaches to facilitating and expediting diagnosis
- Identification of biomarkers that leads to better characterization, management or correlation with outcomes
- Burden of disease (clinical, economic, and/or humanistic burden)
- Epidemiology studies (incl. registries)

Out of scope

- Pediatric studies
- Clinical trials exploring different dosing regimens as currently investigated
- Clinical trials combining Iptacopan with immunosuppressant and anti-C5 treatments
- Head-to-head comparisons
- Studies in other non complement mediated hematologic diseases

CRM

Pelacarsen

Non-drug IITs

Epidemiology associated with elevated Lp(a)

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

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

- Insights on the pro-thrombotic mechanisms impacted by Lp(a)
- Unique features of 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 risk evaluation
- Clinical and economic value of Lp(a) testing

Out of scope

- Comparison / association with LDL-C
- Non-cardiovascular related diseases

Inclisiran (Leqvio)

- ASCVD MOA – atherosclerotic plaque composition/changes
- Real world utilization & implementation of inclisiran post ACS and/or symptomatic PAD
- Population modeling of diverse populations in various health care settings (including HCRU,...)
- Differentiating attributes of inclisiran versus other LLTs (e.g., safety, drug interaction, adherence, outcomes...) in Real World setting
- Effects of inclisiran in high-risk patient population (e.g., diabetes,...)

Iptacopan for Igan Indication (Fabhalta)

- Role of complement system in complement-mediated kidney diseases
- Additional ways to foster diagnosis of glomerulopathies beyond biopsy
- Identification of approaches that lead to better characterization, management or correlation with outcomes in IgAN – e.g. identification of biomarkers, genetic analysis or biopsy-based studies
- Burden of disease (clinical, economic, and/or humanistic burden) – IgAN
- Epidemiology studies (incl. Registries) - IgAN
- Mapping or intervening on the patient journey in IgAN to reduce health care costs or improve patient outcomes

Out of scope

- Pediatric studies (with drug)
- Studies exploring different dosing regimens as currently investigated

Iptacopan for C3G indication (Fabhalta)

- Role of complement system in complement-mediated kidney diseases
- Additional ways to foster diagnosis of glomerulopathies beyond biopsy
- Studies which attempt to clarify the histopathologic complexity/equipoise of C3G

- Identification of approaches that lead to better characterization, management or correlation with outcomes in C3G, ICMPGN, aHUS, LN – e.g. identification of biomarkers, genetic analysis or biopsy-based studies
- Burden of disease (clinical, economic, and/or humanistic burden) - C3G, ICMPGN, aHUS, LN
- Epidemiology studies (incl. Registries) - IgAN, C3G, ICMPGN, aHUS, MN, LN
- Studies on Natural History of C3G and ICMPGN in native vs transplant kidney.
- Mapping or intervening on the patient journey in ICMPGN and C3G to reduce health care costs or improve patient outcomes

Out of scope

- Pediatric studies (with drug)
- Studies including patients with CKD stages 4 and 5
- Head to head studies comparing Ipta to other treatments

Atrasentan for IgAN Indication (Vanrafia)

- Role of the endothelin system in rare renal diseases, including IgAN, FSGS, Alport
- Additional ways to foster (earlier) diagnosis of rare renal diseases, beyond biopsy
- Identification of approaches that lead to better characterization and/or correlation with outcomes in rare renal diseases including IgAN, Alport and FSGS
- Burden of disease (clinical, economic and/or humanistic burden) in rare renal diseases, including IgAN, Alport, FSGS
- Epidemiological studies in rare renal diseases, including IgAN, FSGS, Alport
- Studies evaluating the mechanism of hemodilutional anemia with ERAs

Out of scope

- Pediatric studies (with drug)
- Studies exploring different dosing regimens to those currently being evaluated in Atrasentan CDP
- Studies including patients with CKD stages 4 and 5

Neuroscience
Ofatumumab (Kesimpta)

Multiple Sclerosis

- The experience of use of OMB in sub-populations of RMS (e.g., AA and Hispanic patients, and age)
- The impact of OMB on MS comorbidities and patient-centric outcomes
- The therapeutic role of OMB in MS: Efficacy, safety, tolerability, use in treatment naive patients
- The impact of OMB on both fluid and digital biomarkers in MS
- The MS pathophysiology (including MoA of OMB and its effects on MS pathophysiology) and burden of disease of MS (including impact of OMB)
- The innovative neuroimaging techniques used to measure biomarkers of MS disease/MS inflammation/axonal integrity and function (including effects of OMB)
- The long-term impact on the immune system and long-term safety with B-cell therapies
- Different B-cell depleting therapies have a differential impact on the functioning of the immune system over time, especially on the non-B-cell compartment

Remibrutinib

Multiple Sclerosis

- The impact on CNS – BBB transmigration, microglial impact (activation)
- The impact on biology of progression – PET imaging impact, cognition, fatigue, depression outcomes
- The impact on imaging – SELs, PRLs, cortical lesions impact
- The role for remibrutinib in sequencing of treatments
- The proteome profiling effects of remibrutinib

Myasthenia Gravis

- Impact of remibrutinib on gMG
- Development of biomarkers and endpoint exploration for clinical trial use

Gene Therapy
Zolgensma IV

- Demonstrating or validating care needs for SMA populations post Zolgensma IV Treatment-safety related items
- Value of Zolgensma IV : Cost of care, Quality of life, and Caregiver Burden-Cost effectiveness
- Methods/Processes to assess the efficacy and durability of Zolgensma IV (e.g. bulbar function)
- Biomarkers for efficacy

Out of scope

- Clinical Trials involving Zolgensma IV re-dosing
- Study of Zolgensma IV alternative doses/maximum dose
- Basic Science research that request use of Zolgensma IV

OAV101 IT

- Interventional Studies of OAV101 IT in patients not included in clinical trials (e.g. independently ambulant SMA patients, patients >18 years, severe scoliosis) and patients with AAV9 titers >1:50
- Non-interventional Studies of OAV101 IT assessing sleep, bulbar function, scoliosis and respiratory function, head steadiness and independence.
- Studies on biomarkers assessing clinical response to OAV101 IT

Out of scope

- Clinical Trials involving OAV101 IT re-dosing
- Study of OAV101 IT alternative doses/maximum dose
- Head-to-head comparison with other therapies and combination with other MDT
- Studies of OAV101 IT in patients under 2 years of age.
- Comparative studies between Zolgensma IV and OAV101 IT

Immunology
Ianalumab

- Sjogren's disease US epidemiology
- Sjogren's disease classification and clinical assessment
- Sjogren's disease progression: use of ultrasounds, clinical assessments and or biomarkers
- Sjogren's disease organ domains: generation of evidence in key disease domains
- Sjogren's disease and concomitant conditions (i.e., rheumatoid arthritis, lupus, etc.) and outcomes
- Sjogren's disease in subpopulations (AA, Hispanic, etc.) and outcomes
- Sjogren's disease burden: clinical, social, economical, and humanistic aspects

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