NOVARTIS

IIT US Areas of Interest

Lymphoid

Kymriah

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

Myeloid/Malignant Hematology

Asciminib

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

Non-Malignant Hematology

Iptacopan

With drug:

- Mechanistic studies in PNH
- Studies evaluating complement Factors associated with or predictive of treatment outcome in PNH
- Studies exploring Factor B inhibition in other disease states where complement plays a role

Without drug

- Role of complement system in complement-mediated PNH
- Approaches to facilitating and expediting diagnosis of PNH

- Identification of biomarkers that leads to better characterization, management or correlation with outcomes in PNH
- Epidemiology studies (incl. registries) – PNH

Breast and Gynecological Cancers

Ribociclib

Breast cancer

- Ribociclib in early breast cancer and metastatic breast cancer (limited scope)

Out of scope

- All other studies and tumor types

RLT

[177Lu]Lu-PSMA-617

- Investigating alternative dosing regimens (cycles, frequency) with ^{177}Lu-PSMA-617 in mCRPC
- Radioligand therapy in neoadjuvant setting for localized prostate cancer
- Use of ^{177}Lu-PSMA-617 in adjuvant setting in combination with EBRT + ADT +/- abiraterone in patients with localized prostate cancer post prostatectomy with N1M0 on PSMA PET
- Use of ^{177}Lu-PSMA-617 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 ^{177}Lu-PSMA-617 in combination with other agents in mHSPC or mCRPC
- Treatments up-regulating PSMA expression in prostate cancer
- Use of >6 cycles of ^{177}Lu-PSMA-617 in patients with mHSPC or mCRPC
- Use of ^{177}Lu-PSMA-617 in prostate cancer patients with distinct mutations (e.g., PTEN-loss, AKT, DDR)
- Use of ^{177}Lu-PSMA-617 in patients with low or no PSMA expression in mCRPC
- Safety and efficacy of ^{177}Lu-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

Lutathera / **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 other non-GEP NET SSTR2+ tumors

CVM

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 (e.g. kidney disease, diabetes)

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

- 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
- 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
- Guidance on management of currently modifiable risk factors in the setting of elevated Lp(a)

Out of scope

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

Leqvio

- Mechanistic studies in post ACS/ symptomatic PAD
- Real world utilization & implementation of inclisiran post ACS/symptomatic PAD
- Inclisiran in under-represented patient population (eg, women, pts with treatment disparities in LDL-C lowering, patient types who tend to have worst outcomes)
- Characterization of the effect of inclisiran on lipoprotein metabolism: particle synthesis/secretion, including Lp(a)
- Populaton modeling of diverse populations in various health care settings (including HCRU,...)
- Effects of inclisiran in high-risk patient population (e.g., diabetes,...)
- Differentiating attributes of inclisiran versus other LLTs (e.g., safety, drug interaction, adherence,..)
- Preclinical studies evaluating non-LDL-C-lowering effects

Iptacopan

Iptacopan for Igan Indication

- 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 regiments as currently investigated
- Any study, which combines iptacopan with immunosuppressant

Iptacopan for C3G indication

- 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

Atrasentan

Atrasentan for IgAN Indication

- 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

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

In scope

- 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
- Basic Science research that request use of OAV101

OAV101 IT

In scope

- Interventional Studies of OAV IT in patients not included in clinical trials (e.g. ambulant SMA patients, patients >18 years, 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
- Studies of OAV IT in patients under 2 years of age.

Dermatology

Cosentyx

- Approaches to facilitating and expediting diagnosis and treatment of HS to modify disease progression
- Development of novel imaging techniques to investigate the role of secukinumab in limiting progression of HS and PsO
- Machine learning techniques to create predictive models for disease trajectories, and IL-17A inhibition responses across disease subpopulations (e.g. disease phenotypes, Black/African American, super-responders, etc.)
- Essential factors (such as biomarkers) for predicting disease and treatment outcomes
- Burden of disease (clinical, economic, and/or humanistic burden) – HS and PsO
- Development and validation of scoring tools and patient reported outcomes in HS

Out of scope

Dermatologic conditions other than psoriasis and hidradenitis suppurativa

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 (i.e., rheumatoid arthritis, lupus, etc.)

Cosentyx, Illaris, and Zortress

IITs in these products are no longer supported
