Therapeutic Areas of Interest

Education and research opportunities may exist for topics listed below, including but not limited to:

Critical Care

Acute Hypertension/ Cleviprex (clevidipine)

- **Education:**
  - Blood Pressure management in acute stroke (ischemic and hemorrhagic)
  - Blood pressure management in neuro-interventions
  - Blood pressure variability and correlation to clinical and quality outcomes
  - Peri-operative (pre, intra, post) blood pressure management in cardiac and/or vascular surgical procedures
- **Research:**
  - Cleviprex performance in Blood Pressure management in acute stroke (ischemic and hemorrhagic) presentation
  - Cleviprex performance in reducing blood pressure variability
  - Cleviprex performance related to clinical, operational or quality outcomes
  - Cost-effectiveness of Cleviprex for the reduction of blood pressure when oral therapy is not feasible or not desirable

Antiplatelet/Kengreal (cangrelor)

- **Education:**
  - Pharmacological considerations for complex and high-risk PCI, including cardiogenic shock and cardiac arrest
  - P2Y12 Inhibitor treatment strategies in acute coronary syndrome patients undergoing PCI
  - Perioperative risk and antiplatelet management in patients undergoing surgery
  - Role of antiplatelet therapy in acute interventions for ischemic stroke
- **Research:**
  - Cost-effectiveness of IV Kengreal in complex PCI and/or high-risk patient presentations
  - Clinical and pharmacoeconomic outcomes of IV Kengreal from large, real-world database
  - Safety and efficacy of IV Kengreal in the setting cardiogenic shock and cardiac arrest
  - Transition from IV Kengreal to oral P2Y12 inhibitor therapy in patients who have previously received a P2Y12 inhibitor prior to PCI
  - Role of IV Kengreal in acute neuro-endovascular stenting for ischemic stroke

Special Care

Neonatal Respiratory Distress Syndrome (nRDS)

- **Education:**
  - Early rescue surfactant therapy for neonates with nRDS
  - Simulation training on skills such as: intubation, resuscitation, and surfactant therapy administration
  - Quality Improvement: Early respiratory management and/or "Golden Hour" protocols for infants born at risk for, or who have developed, nRDS; preventative strategies aimed to reduce the incidence of BPD

- **Research:**
  - Improvement of early identification of infants in need of surfactant therapy (within first 2 hours of life), based on clinical indicators (e.g. FiO_2) or other novel/innovative methods
  - Characterization of short and long-term outcomes for early ($FiO_2 \geq 0.3$) vs. later administration of surfactant
  - Novel and innovative methods to administer surfactant therapy
  - Surfactant therapy in late preterm neonates ≥ 32 wGA and its impact on RDS outcomes

Rare Disease

Inborn Errors of Metabolism

- **Fabry Disease**
  - **Education:**
    - Early diagnosis for Fabry Disease, increased awareness of disease characterization
    - Optimized assessment of disease progression in Fabry patients
    - Unmet needs in the current management of patients with Fabry disease
    - Fabry disease manifestations in female patients
  - **Research:**
    - Innovative approaches to increasing the diagnosis rate for FD
    - Clinical or biomarker evidence supporting shorter time to treatment initiation with the goal of improving long-term patient outcomes
    - Optimized assessment of disease progression in treated patients
    - Validation of new biomarkers of subclinical and clinical disease progression
    - Further understanding of the immunogenicity and bioavailability profile of PRX-102
- **Alpha Mannosidosis**
  - **Education:**
    - When to suspect alpha-mannosidosis – clinical and laboratory parameters
    - Current management of alpha-mannosidosis
  - **Research:**
    - New approaches to alpha mannosidosis screening and diagnosis
    - Characterization of the clinical and immunological profile of patients with alpha mannosidosis
    - Understanding the natural history of alpha mannosidosis
    - Characterization of disease monitoring practices in the real-world setting
    - Biomarkers and clinical monitoring of disease progression and treatment effect
    - Understanding current treatment approaches for alpha mannosidosis
    - (Specific to countries where Lamzede is approved)
      - Experience with Lamzede in the real world
      - Impact of Lamzede on patient-centric outcomes, burden of disease, and quality of life

Rare Hematology and Immunology

- **Hematology** (Therapeutic areas include hemoglobinopathies such as Sickle Cell Disease, Beta Thalassemia and other rare anemias)
  - **Education:**
    - General iron overload and damage
    - Iron chelation therapy in thalassemia, SCD, and other rare anemias
  - **Research** (all studies requiring product are to be done with Twice-A-Day formulation):
    - Use of Ferriprox Twice-A-Day (TAD) formulation
    - Ferriprox use in sickle cell disease (SCD) and/or thalassemia patients
    - Combination therapy with iron chelation therapy (ICT)
    - Ferriprox use in other hematological diseases, including rare anemias. Examples include aplastic anemia, myelodysplastic syndrome (MDS), hematochromatosis.
- **Immunology** (Adenosine Deaminase Severe Combined Immunodeficiency (ADA-SCID))
  - **Education:**
    - SCID disease state and available pharmacotherapies
    - Management of primary immunodeficiencies and SCID

- **Research:**
  - Understanding SCID: mechanism of disease and complications
  - Clinical outcomes in enzyme replacement therapy (ERT)-treated ADA-SCID patients
  - Biomarkers and clinical monitoring of ADA-SCID
  - Long term outcomes and survival of ADA-SCID patients