Investigator-Initiated Research: Areas of Interest

Updated June 2018

Rare Disease

Qualified researchers are invited to submit investigator-initiated research (IIR) proposals, according to the guidance and instructions found on the Pfizer IIR portal at www.Pfizer.com/IIR. All proposals must be submitted via the IIR submission portal at: https://iirsubmission.pfizer.com. An IIR proposal requesting Pfizer support (e.g., funding and/or drug supply) is not a guarantee of acceptance or approval of that proposal. Decisions on support for IIR submissions are made by the applicable Pfizer Global Review Committee. A formal notification regarding the status of your application will be sent once a decision is reached. Pfizer support will only be extended upon the execution of an IIR agreement. For any questions, please send an email to IIR@pfizer.com.

Endocrine : pegvisomant

Research areas to be considered for Pfizer support include:

- Morbidity and mortality in patients with Acromegaly
- Novel strategies including Quality of Life and Patient Reported Outcomes to evaluate and treat acromegaly or children with growth disorders
- Early diagnosis and treatment of Acromegaly; consequences of late diagnosis/treatment initiation

PLEASE NOTE THAT DUE TO A SCHEDULED SYSTEM UPDATE, NO NEW APPLICATIONS WILL BE ACCEPTED BETWEEN OCTOBER 1 AND DECEMBER 31

Gaucher Disease: taliglucerase

Research areas to be considered for Pfizer support include:

- Early treatment of Gaucher Disease; novel/alternative dosing paradigms; effects of Gaucher Disease such as: pulmonary, bone, cognition/cognitive decline, Quality of Life (QoL), immunology and mechanisms of cellular uptake, bone uptake and crossing barriers (e.g. blood-brain barrier).
- Studies that explore Efficacy endpoints
  - improvement in pulmonary and/or bone status, QoL, use in type 3 Gaucher disease
  - immunogenicity, allergic reactions

Target population: Children and adults of any age, gender

Hemophilia

Research areas to be considered for Pfizer support include:

- Basic Science of Gene Therapy for Hemophilia
  - Basic science, tropism, transduction efficiency & tolerability of Adeno-associated virus
  - AAV antibody Titer assessment, reduction, tolerance
  - Role of immunosuppression in managing transaminitis

- Basic Science of TFPI & Anti-TFPI Monoclonal Antibodies
  - Regulation of Coagulation
  - Basic biology of TFPI interactions with Protein C, ATIII, & Protein S
  - Cross talk among regulators (e.g. Protein S being a co-factor for both Protein C and TFPI)
  - Role of different TFPI pools in regulation of coagulation
  - Pharmacology resulting from concomitant treatments (especially antifibrinolytics) added to anti-TFPI

- Patients with MILD Hemophilia A or B
  - Natural history of mild Hemophilia
  - Arthropathy: presence, development, clinical burden & Joint damage in mild Hemophilia
  - Quality of Life/Work analysis in mild Hemophilia
  - Clinical profile & healthcare utilization in mild Hemophilia
  - Cost of Care, including non-hemophilia related healthcare utilization in mild Hemophilia

Sickle Cell Disease (SCD)

Research areas to be considered for Pfizer support include:

- The pathophysiology of sickle cell disease and/or including biomarkers
- Natural history of SCD
- Epidemiology of SCD
- SCD care pathways including: standards of care, access/barriers to care, translation of patient reported outcomes (PRO)
- Point-of-care testing for SCD

Transplant: sirolimus

Research areas to be considered for Pfizer support include:

- Preservation of renal function in kidney transplant patients
- Reduction of post-transplant malignancy
- Reduction of post-transplant viral infections
- Improvement in understanding the management of side effects
- Exploration of the use of sirolimus beyond kidney transplantation

TTR Amyloidosis: tafamidis

Research areas to be considered for Pfizer support include:

- Early identification, evaluation, diagnosis & treatment
  - e.g.,Biomarker studies
- Epidemiology
- Post organ transplant
  - Use of tafamidis
  - Natural course of disease
- Non-Val30Met genotypes
- Late-onset disease (onset after 50 years of age)
- Mixed phenotypic manifestations (e.g. polyneuropathy and cardiomyopathy)
- Use of tafamidis in the clinical setting (i.e. real world evidence)
- Functional role of TTR in humans or non-human primates

We are not currently accepting proposals focusing on:

- Head to head studies
- Any indications outside of TTR amyloidosis
- Pediatric investigations
- End stage disease (Stage 3-4 TTR amyloidosis)
- Dose response studies
- Animal studies (except requests for pure substance only)