Request for Applications to Establish New Programs of Excellence in Orphan Diseases

The Orphan Disease Center (ODC) of the Perelman School of Medicine is requesting applications from Penn and CHOP faculty for seed funding of $100,000 per award to evaluate the feasibility of establishing new Programs of Excellence (POEs).

One mechanism by which the ODC supports orphan disease research is through its POEs which at present include programs in Lysosomal Storage Diseases, CDLk5 Deficiency, Motor Neuron Diseases and Liver Metabolic Diseases. Each POE is designed to further the mission of the ODC which is: “to develop transformative therapies using platform technologies that can be deployed across multiple rare diseases. We will emphasize disorders with substantial unmet need independent of their incidence and will strive to assure access to patients of all populations.”

Important features of a POE are:

- It involves a disease or clusters of diseases with significant unmet need
- Has the potential to achieve substantial treatment effects – i.e., there exists tractable therapeutic targets that could restore function and/or stabilize a degenerative process
- ODC is well-positioned to help fill critical gaps in translational research
- Is a “common” orphan disease or a member of a disease cluster in which common therapeutic platforms (i.e., therapeutic antibodies, stem cells, gene therapy, RNAi, genome editing, etc.) can be applied across members of the cluster
- There is a viable strategy for securing sustainable funding
- There exists a substantive PENN and/or CHOP research connection and/or a connection through its alumnae, benefactors, faculty, staff and/or students

The goal of this RFA is to provide faculty with resources to evaluate the feasibility of establishing new POEs. These resources can be used in whatever way that will be useful in the further development of a new POE concept including but not limited to: hosting a symposium, hiring consultants, conducting pilot research studies, progressing existing research to a value inflection point to enhance potential biopharma collaboration, creation of critical research tools, etc. It is hoped that work done under the auspices of these pilot programs will better inform ODC leadership about the creation of new POEs.

The first step is for faculty to submit a one-page Letter of Intent (LOI) to Samantha Charleston by Wednesday, September 7, 2016. Applicants will be notified of the outcome of the initial review by Monday, September 26 and potentially meritorious LOIs will be asked to submit a 5-page proposal due Monday October 24 with the goal of funding to begin January 1, 2017. The awards are for $100,000 direct costs and should span no longer than 12 months. Review and oversight of the LOIs and the full applications is conducted by the Steering and External Advisory Committees of the ODC with input provided, as necessary, by topic experts. The one page LOI should be structured as follows:

- List PI and co-PIs and affiliated institutions with departments. Inclusion of collaborators outside of Penn and CHOP is encouraged where appropriate.
- Describe the disease and/or disease cluster including: information about unmet need, prevalence/incidence, and potential for substantial treatment effects. We will favor proposals that create new opportunities rather than fund existing, well-organized orphan disease programs.
- What important gaps would a new POE be in a position to fill?
- Describe potential mechanisms for securing resources to grow and sustain a new POE. This could include philanthropy, biopharmaceutical collaborations, government grants/contracts, and/or foundation support.
- How will the $100,000 pilot funding be used and what will be the deliverables at the completion of the award?