**Creating Champions For Open Source Rare Disease Drug Discovery With An App**

Sean Ekins1,2,3,4,5,6,7, Jill Wood2, 8, Lori Sames4, 9, Allison Moore3 and Alex M. Clark

1Collaborations in Chemistry, 5616 Hilltop Needmore Road, Fuquay Varina, NC27526, USA.

2Phoenix Nest, P.O. BOX 150057, Brooklyn NY 11215, USA.

3Hereditary Neuropathy Foundation, 432 Park Avenue South – 4th floor,New York, NY 10016, USA.

4BioGAN Therapeutics, P.O. Box 130, Rexford, NY 12148, USA.

5Department of Pharmaceutical Sciences, University of Maryland, 20 Penn Street, Baltimore, MD 21201, USA.

6Department of Pharmacology, University of Medicine & Dentistry of New Jersey (UMDNJ)-Robert Wood Johnson Medical School, 675 Hoes lane, Piscataway, NJ 08854, USA.

7Division of Chemical Biology and Medicinal Chemistry, UNC Eshelman School of Pharmacy, University of North Carolina at Chapel Hill, NC 27599-7355, USA.

8Jonah's Just Begun, P.O. Box 150057, Brooklyn, NY 11215, USA.

9Hannah's Hope Fund, P.O. Box 130, Rexford, NY 12148, USA.

10Molecular Materials Informatics, 1900 St. Jacques #302, Montreal, Quebec, Canada H3J 2S1

**Abstract**

To date only a few hundred of the 7000 rare diseases have treatments. The current rate of knowledge creation, drug discovery, development and regulatory approval is inadequate for the growing rare disease patient population and to fill the treatment gap. Emboldened by the Open Source software movement, neglected disease research has recently adopted open source drug discovery as an approach to accelerating the research for tuberculosis and malaria. We believe rare disease research can also benefit from the open source drug discovery principles. For example, our own difficulty in accessing published information on Sanfilippo syndrome and sharing with others, inspired the development of a mobile app to collect social media and open information from the internet on this and other rare and neglected diseases. The development of Open Drug Discovery Teams (ODDT) has in turn led to increased opportunities to raise awareness (e.g. additional lysosomal diseases, Giant Axonal Neuropathy, Charcot-Marie-Tooth Disease etc) and the need for more open, collaborative research on rare diseases. However there are still significant challenges as many organizations want closed systems to preserve their intellectual property. We need to create champions for open source rare disease drug discovery and these can be assisted by apps. There need to be tools that can take some weight of the parent/patient and help make sense of the rare disease literature, foster collaboration and leverage what is learnt from other rare diseases. In addition there need to be coordinated efforts to help find researchers funding or projects they should work on and in turn help parents find scientists to solve their problems. This leverages crowdfunding, crowdsourcing and open innovation elements which can be readily incorporated into an app. The benefits of open source rare disease drug discovery may be increased visibility for researchers, less repetition and faster progress towards treatments.