World Orphan Drug Congress – Repurposing Existing Medicines

Boston, MA - May 28, 2024 - Myles Axton, PhD, Chief Genetics Researcher at Luminous Mind, Inc., attended the recent World Orphan Drug Congress and shared some of the key messages from the Congress.

The Orphan Drug Act of 1983 (ODA) has been a success, resulting in 600 drugs for 1200 disease indications.

Indeed, just under half of FDA drug approvals between 2017 and 2021 were for drugs used to treat rare conditions. The ODA provides incentives, including designation and seven years of market exclusivity, for developers of drugs for conditions affecting fewer than 200,000 people in the US, or drugs for which the manufacturer would not expect to recover the costs of research and development without this protection.

Luminous Mind Inc. is currently focusing on repurposing safe and effective small molecules as therapies for several genetically defined conditions. From the patient perspective, therapy is successful when it prioritizes the treatment of the symptoms that most distress the patients and their supporters. Consequently, while our repurposing program integrates genetic, clinical, and pharmaceutical data, we are also reaching out to patient advocates to help us understand the shared differences in individual symptoms and experiences that predict treatment success for each of these therapeutic options.

Louis Herlands, CEO of Luminous Mind, emphasizes, "Our mission includes developing impactful medicines for rare diseases, leveraging our team's unique expertise and the latest advances in genomics. We are well-positioned to make significant strides in this field." Herlands adds, “Our team's combined expertise in genomics and drug development strengthens our capability to address Central Nervous System (CNS) rare diseases. We are well-equipped to navigate the complexities of developing treatments for rare genetic conditions."

A key asset to Luminous Mind’s success is Dr. Bert Spilker, Head of Clinical and Regulatory Affairs. Dr. Spilker's 15 textbooks on drug development are required reading in the pharmaceutical industry. He was the Senior Vice President of Scientific and Regulatory Affairs for PhRMA and President and Co-Founder of Orphan Medical, Inc., a pioneering company that brought seven drugs for patients with uncommon diseases to market in just five years. While Orphan Medical achieved remarkable success, the advances in genomics and the diagnosis of rare genetic diseases available today provide us with tools and insights that were not in place during that era.

During the World Orphan Drug Conference, one such patient advocate that was featured was Julia Taravella of the Rare Trait Hope Fund. As an oil industry manager, she took an engineer's approach to a rare degenerative neurological and immune system disorder in her family. The family funded proof-of-principle gene therapy efficacy studies in mice to establish the therapy with the FDA as an Investigative New Drug. With superior analytical skills, she was able to explain that rare diseases vary greatly in their rarity, and just how rare their family variant is: it is only the tenth time this particular gene has been seen in this disease worldwide.

Taravella continued, citing analysis by the Orphanet database, which explains that about 80% of rare disease patients have one of just 149 rare diseases, each with a population frequency greater than one per ten thousand people. In contrast, at the ultra-rare end of the mutation spectrum—totaling just a third of a percent of rare disease patients—there is a much wider range of over 3,000 diseases, each rarer than one in a million in their population.

Bespoke gene editing is an approach to meet the needs of ultra-rare genetic conditions. However, Peter Marks of the FDA Center for Biologics Evaluation points out, "The vast number of variants and gene targets is huge, so we want to have a platform of delivery" to reduce the number of new parts requiring individual testing for safety and efficacy. He believes that even common diseases such as diabetes and coronary artery diseases will eventually be treated by gene editing of rare gene variants.

Geneticist Sarah Glass commented that we have over 35 years of therapy experience with RNA sequences and know which parts of the molecules cause adverse immune reactions. This experience can be utilized in the design and synthesis of RNA sequences that will guide gene editing. Adding to Marks' view that a common platform is achievable, she points out that the gene therapy strategy approved for sickle cell targets the same hemoglobin molecule in the blood of people with other hereditary anemias (called thalassemias—sea blood—because of their prevalence in populations around the Mediterranean Sea).

Gene editing for neural and psychiatric conditions would be a challenge orders of magnitude greater than the task of gene editing diseases of stem cells, blood, and bone marrow. Although we do not yet have reliable technologies for precision targeting of billions of specific neurons among the trillions in a brain that is already wired up and functioning, there are many specific small molecules that have great specificity. Functional diseases of the mind and brain, including inflammatory and metabolic subtypes of depression, are common; but these common diseases can be divided and conquered. Analyzed genetically, each disorder will inevitably be reduced to a set of more clearly defined conditions, each to be addressed with a more targeted therapy.

Repurposing existing medicines for new indications may meet an ambitious goal of a thousand new drugs approved by 2027, according to Oxana Iliach of the International Rare Diseases Consortium (IRDRC). Iliach gave the example of iloprost, a repurposed vasodilator drug approved by the FDA this year to prevent finger and toe amputation in severe frostbite. This drug was originally approved by the FDA two decades ago for pulmonary artery hypertension. She says the IRDRC roadmap starts with stakeholder mapping, matching patient needs with available information about existing drug profiles. She recommends prioritizing acceptability to the FDA or the EU EMA, as other regulatory authorities have readily aligned their standards. In any case, there are many safe drugs already approved. The FDA's Centers for Drug and Biologics Evaluation have previously approved 140,000 drug-indication combinations, totaling 12,699 small molecules and 3,884 biologics.

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About Luminous Mind, Inc.

Luminous Mind Inc., with its focus on Central Nervous System (CNS) disorders, applies a rigorous therapeutic discovery and development process that includes the repurposing of existing, abandoned, and developmental compounds for new indications. Groundbreaking scientific insights in neuroscience, genetics, systems biology, and brain circuitry, together with powerful new tools drive its discovery efforts. For additional information, please visit www.LuminousMindInc.com.

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