A Fundamentally Different Approach to Rare Diseases

We’re leveraging our nearly 15 years of experience pioneering leadership in the field of cellular metabolism to raise the bar when it comes to how rare diseases are viewed and treated. Diseases are typically considered rare if they affect fewer than 200,000 people in the United States. These diseases are likely to be underdiagnosed given the lack of available therapies or diagnostics, the rarity of the condition, or limited understanding of how the disease genetics relate to disease phenotype. Many of these diseases are severe or life-threatening, and current treatment options for these disorders are generally limited. At Agios, we’re pushing beyond incremental improvements to develop transformative small molecule medicines that have the potential for long term, disease-modifying effects.

Our ability to rapidly advance new therapies for people with rare diseases is propelled by our unique understanding of cellular metabolism. Cellular metabolism is a complex biological process that facilitates many of the mechanisms required for cellular division, growth, and function. Our leadership in this field has provided evidence that dysregulation of normal cellular metabolism plays a crucial role in many rare diseases. Our novel small molecule approach aims to correct the defects within diseased cells, bringing relief to the people, and their loved ones, living with severe conditions.
Mitapivat (PK Activator)

Mitapivat is a wholly owned, first-in-class, oral activator of both wild-type (healthy) and mutated pyruvate kinase (PK) enzymes, which play a critical role in red blood cell health, energy and lifespan. Agios is conducting pivotal studies of mitapivat in alpha- and beta-thalassemia, sickle cell disease and pediatric PK deficiency.

AG-946 is a potent investigational oral activator of both wild-type and mutated PK enzymes. Agios is studying AG-946 in a Phase 1 trial in healthy volunteers and sickle cell disease and in a Phase 2a/b study in anemia due to lower risk myelodysplastic syndromes (LR-MDS).

Preclinical Programs

Since our founding, Agios has been a pioneering leader in cellular metabolism, advancing therapies for patients with unmet needs. Agios’ research team is focused on advancing a phenylalanine hydroxylase (PAH) stabilizer for the treatment of a rare, inherited disease known as phenylketonuria (PKU).