

Quick Glance

Founded: 2008

IPO: July 2013

Ticker Symbol: AGIO

Vision

AgiOS is passionately committed to applying our leadership in the field of *cellular metabolism* to transform the lives of patients with *rare diseases*.

Values

Aim High

Come Together

Embrace Differences

Bring Your Whole Self

Blaze New Trails

Leadership

Brian Goff

Chief Executive Officer

Cecilia Jones

Chief Financial Officer

Jim Burns

Chief Legal Officer

Sarah Gheuens, M.D., Ph.D.

Chief Medical Officer,
Head of R&D

Ellen Lopresti

Chief People Officer

Tsveta Milanova

Chief Commercial Officer

Charlie Newman

Chief Business Officer

Clive Patience, Ph.D.

Chief Technical
Operations Officer

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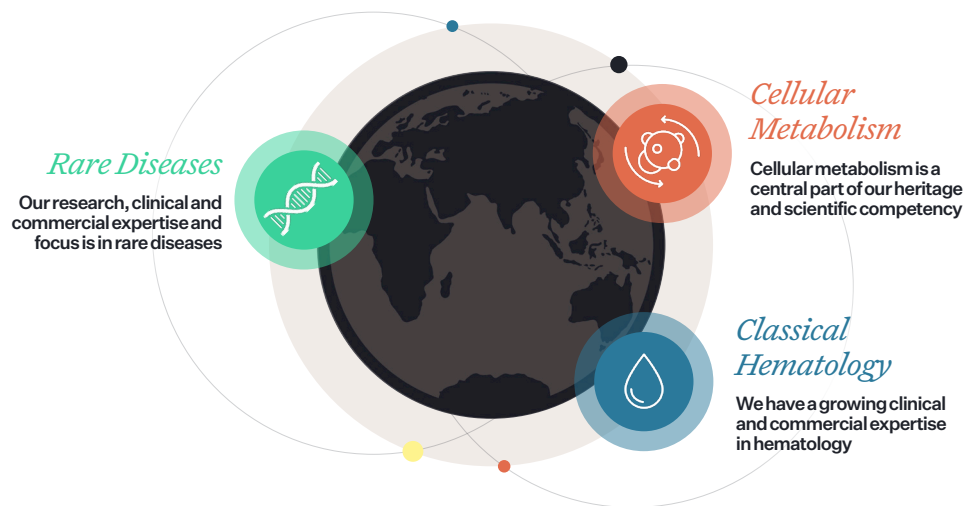
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Fueled by *connections.*

Building Connections, Pioneering Therapies

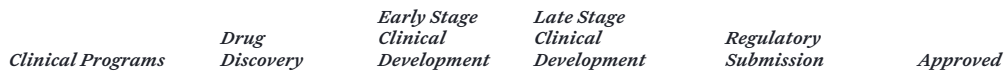
AgiOS is a biopharmaceutical company that is fueled by connections. We are passionately committed to applying our leadership in the field of cellular metabolism to create life-changing therapies for people living with rare diseases. The bonds we build with patient communities, healthcare professionals, partners and colleagues help elevate our thinking and propel our science to new heights. These connections, combined with our cellular metabolism expertise, have fueled the development and approval of our first-in-class pyruvate kinase (PK) activator, as well as other investigational therapies.



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)

	Drug Discovery	Early Stage Clinical Development	Late Stage Clinical Development	Regulatory Submission	Approved
Adult PK Deficiency	Progressing	Progressing	Progressing	Progressing	APPROVED in the U.S., Great Britain and EU
			ACTIVATE and ACTIVATE-T		
Non-transfusion Dependent Adult Thalassemia	Progressing	Progressing	Progressing		
			ENERGIZE		
Transfusion Dependent Adult Thalassemia	Progressing	Progressing	Progressing		
			ENERGIZE-T		
Adult Sickle Cell Disease	Progressing	Progressing	Progressing		
			RISE UP		
Pediatric PK Deficiency – Regularly Transfused	Progressing	Progressing	Progressing		
			ACTIVATE-kidsT		
Pediatric PK Deficiency – Not Regularly Transfused	Progressing	Progressing	Progressing		
			ACTIVATE-kids		
Pediatric Thalassemia	Progressing	Progressing			
		Planning underway			
Pediatric Sickle Cell Disease	Progressing	Progressing			
		Planning underway			

AG-946 (PK Activator)

	Drug Discovery	Early Stage Clinical Development	Late Stage Clinical Development	Regulatory Submission	Approved
MDS-associated Anemia	Progressing	Progressing			
		Phase 2			
Healthy Volunteers/ Sickle Cell Disease	Progressing	Progressing			

Preclinical

	Drug Discovery	Early Stage Clinical Development	Late Stage Clinical Development	Regulatory Submission	Approved
Phenylalanine Hydroxylase (PAH) Stabilizer	Progressing				
Other PKR/PKM2 Development Candidates	Progressing				

Clinical Programs

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).

Cautionary Note Regarding Forward-Looking Statements

This fact sheet contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development programs including mitapivat and AG-946; the potential benefits of Agios’ product candidates; and the potential benefit of its strategic plans and focus. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “would,” “could,” “potential,” “possible,” “hope” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Agios’ current expectations and beliefs. For example, there can be no guarantee that any product candidate Agios is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios’ product candidates will successfully continue. There can be no guarantee that any positive developments in Agios’ business will result in stock price appreciation. Management’s expectations and, therefore, any forward-looking statements in this fact sheet could also be affected by risks and uncertainties relating to a number of other important factors, including, without limitation: risks and uncertainties related to the impact of the COVID-19 pandemic to Agios’ business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; Agios’ results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA, the EMA or other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Agios’ ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios’ ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios’ ability to maintain key collaborations; and general economic and market conditions. These and other risks are described in greater detail under the caption “Risk Factors” included in Agios’ public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this fact sheet speak only as of the date of this fact sheet, and Agios expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.