

Amicus Therapeutics K.K.

Amicus Therapeutics is a global, patient-dedicated biotechnology company that focuses on the discovery, development, and commercialization of medicine for rare diseases that fundamentally change people's lives with extraordinary science.

Amicus Therapeutics K.K., a Japanese subsidiary of the company, celebrates its third anniversary, and has recently moved to a larger office in the center of Tokyo in anticipation of further growth and success.

Amicus Therapeutics (herein after "Amicus") developed and commercialized its first product Galafold® (migalastat), an orally-administered, small-molecule drug known as a pharmacological chaperone, for the treatment of Fabry disease. Fabry disease is an inherited lysosomal disorder caused by deficiency of the enzyme alpha-galactosidase A, leading to the accumulation of specific lipids in the affected tissues, including heart, kidneys, and skin. The accumulation of lipids and the progressive deterioration of organ function inherent to Fabry disease is believed to lead to morbidity and mortality.



Extraordinary measures to save children with intractable disease

The origin of Amicus dates to when the two children of John Crowley, Chairman and CEO, developed Pompe disease with a life expectancy of only 5 years. To save his children's life, Crowley became President & CEO of a biotechnology company that was researching a treatment for Pompe disease. After many twists and turns, John's children were able to participate in the clinical study of an investigational drug. As a result, his two children survived and have now reached adulthood. In 2002, John assumed an executive

role at Amicus, later becoming Chairman and CEO of the company. The Hollywood movie *Extraordinary Measures*, released in 2010, is based on the story of the Crowley family and encourages patients and families with intractable diseases around the world. In Japan, Fuji TV reported the story as part of its popular program *Unbelievable* in July 2010, making the family's story, and Pompe disease, widely known in this country as well.

Amicus is at the leading edge of novel treatments for rare and orphan diseases



In November 2018, Amicus received two awards. First, Amicus' Galafold® won one of the two top prizes at the UK Prix Galien, the biennial awards which recognize the most innovative and high impact new treatments for patients. UK Health minister Lord OShaughnessy presented the award to Amicus. Second, the Penn Center for Innovation (PCI) named the Amicus-University of Pennsylvania partnership the Deal of the Year.



Galafold®

A new story in rare disease is unfolding

In addition to Galafold®, AT-GAA, an investigational therapy for the treatment of Pompe disease, is currently in late stage development. Pompe disease is a genetic disorder in which glycogen (a complex sugar) builds up in the body's cells. This buildup occurs because there is a deficiency of the acid alpha glucosidase (GAA) enzyme, which is responsible for breaking down glycogen in the body.

Ever committed to bettering the lives of those with life-threatening rare and orphan diseases, Amicus has a strong pipeline for additional treatments for other diseases including Batten disease, Mucopolysaccharidosis, and more.

Mr. Chris Hartz (General Manager, Amicus Therapeutics K.K.) says: "We believe our success is measured by the number of lives we can impact. For that we encourage and embrace constant innovation."

Amicus enters the Japanese rare disease market with the establishment of its subsidiary

In 2016, Amicus established its Japanese subsidiary in Tokyo and has more recently expanded to a larger office.

"Establishing an entity in Japan without any local partners was a huge challenge for a young company like us. When the Japan entity was formed, our first product Galafold® had only been approved in the EU 6 months previously. However, Amicus believed direct entry was the best way to bring our product, along with our unique corporate culture, to patients in Japan," commented Chris Hartz (General Manager, Amicus Therapeutics K.K.).

The origin of chaperone therapy is as a result of the research by a Japanese doctor, Yoshiyuki Suzuki and Mr. Hartz went on to comment: "We are very proud that Japanese Fabry patients now receive the benefits of Dr. Suzuki's initial research. We also hope to have many future

opportunities to deliver new medicines for people living with rare metabolic diseases in Japan."

Building on our experience and strengthening our commitment to the rare disease community in Japan

In August 2019, Amicus announced a licensing agreement with Ultragenyx Pharmaceuticals, Inc. for the development and commercialization of Mepsevii™ for Mucopolysaccharidosis VII in Japan. Ultragenyx markets the drug in the US, EU, and Brazil but not in Japan. "With no other company bringing this drug to Japanese patients, we take it as our duty to deliver this for even the small number of the patients," Mr. Hartz says.



Mr. Chris Hartz (General Manager, Amicus Therapeutics K.K.)

Amicus gains momentum worldwide

In February 2019, Amicus established the Global Research and Gene Therapy Center of Excellence in Philadelphia, PA. This center will advance Amicus' commitment to world-class science that makes a meaningful difference in the lives of people living with rare metabolic diseases.

In May 2019, Amicus and the Perelman School of Medicine at the University of Pennsylvania announced a major expansion to their collaboration with rights to pursue collaborative R&D of novel gene therapies for lysosomal storage disorders and 12 additional rare diseases. The collaboration has been expanded from three to six programs for rare

genetic diseases and now includes: Pompe disease, Fabry disease, CDKL5 deficiency disorder, Niemann-Pick Type C, Mucopolysaccharidosis Type IIIB and Type IIIA, both also known as part of Sanfillipo Syndrome.

The deal has provided Amicus with the largest portfolio of gene-therapy products for rare diseases in development, of any company in the world.



JETRO's support

For the establishment of Amicus' Japanese subsidiary, the JETRO Invest Japan Business Support Center (IBSC) offered consultation on market and regulatory matters, referred Amicus to service providers including bilingual recruitment agencies which specialize in the medical field, and provided market information.

Regarding JETRO's support, Mr. Chris Hartz said, "JETRO provided such a high level of information and the requirements needed to set up the local entity. JETRO's support saved time and resources, which was imperative for a start-up company like us who needed to act fast."

(Interviewed October 2019)

Company History

2002	Founded in Cranbury, NJ	2018	Galafold® approval and launch in Japan and the US
2005	John Crowley appointed as Chairman & CEO		
2007	Listed on the NASDAQ	2018	Entered R&D collaboration with the University of Pennsylvania on gene therapies
2016	Galafold® approval and launch in EU	2019	Operates in 27 countries with about 600 employees
2016	Amicus Therapeutics KK established in Japan		

Amicus Therapeutics K.K

Establishment:	November 2016
Business overview:	Development and commercialization of treatments for rare and intractable diseases
Parent Company:	Amicus Therapeutics UK Ltd.
Address:	Shin Marunouchi Center Building 19F, 1-6-2 Marunouchi, Chiyoda-ku, Tokyo 100-0005
URL:	http://www.amicusrx.jp/

Support from JETRO

- Consultation on market and regulatory matters
- Referrals to service providers (bilingual recruitment agencies which specialize in the medical field)
- Provision of market information