New treatment for Fabry disease to be listed on Australia's Life Saving Drugs Program

From 1 November 2018, Galafold® (migalastat) will be listed on Australia's Life Saving Drugs Program (LSDP) for the long-term treatment of patients 16 years and older with a confirmed diagnosis of Fabry disease, who have an amenable mutation.[1]

- *Fabry disease is a rare, genetic, debilitating disease that is thought to affect between 1 in 40,000 to 1 in 117,000 people.*[2]
- *Galafold is a first-in-class, oral, precision (genetically targeted) medicine and is the first new therapy to be approved in Australia for the treatment of Fabry disease in over 12 years.*[1,3]
- *Amicus Therapeutics Pty Ltd (Amicus) officially announces the launch of its Australian operations.*

SYDNEY, AUSTRALIA | 1 November 2018 – Amicus Therapeutics (Nasdaq: FOLD) has today welcomed the listing of its oral, precision medicine, Galafold® (migalastat), on Australia's Life Saving Drugs Program (LSDP) for the long-term treatment of patients aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. Galafold is the first and only oral precision medicine available for the treatment of Fabry disease, and has been approved for use in Australia by the Therapeutic Goods Administration (TGA) since 15 August 2017.[1,4]

"The listing of Galafold on the LSDP is welcome news as it means there is now a new treatment option for patients who have amenable Fabry mutations," said Associate Professor Kathy Nicholls, Consultant Nephrologist and Lead Physician for Metabolic Disorders at Royal Melbourne Hospital Nephrology Unit.

"As an oral medication, Galafold offers a convenient and viable alternative to fortnightly infusions of enzyme replacement therapy for some patients with Fabry disease. The science behind this new medicine is really exciting and has made us think about the treatment of Fabry disease in a completely different way. It moves us closer to personalised therapy for Fabry disease."

Fabry disease is a rare, genetic and potentially life-threatening disease, belonging to a group of inherited conditions called lysosomal storage disorders.[5] People with Fabry disease are missing, or do not have enough of, the functioning enzyme alpha-galactosidase A (alpha-GAL A), which is responsible for helping the body to break down the fatty substance globotriaosylceramide, or GL-3.[5] As a result of this enzyme deficiency, GL-3 can build up in the body and impact key organs, such as the kidneys and heart, potentially leading to multi-organ failure including kidney failure, heart attack and stroke.[5]

As a precision, or genetically targeted, medicine, Galafold works by stabilising the body's own dysfunctional enzyme, so it can clear the accumulated GL-3 in patients who have amenable mutations. An amenable mutation is one that is responsive to therapy with Galafold, based on a test called the *Galafold Amenability Assay*.[6] The Galafold Amenability Assay is not a separate diagnostic test. The Assay determines which mutations will respond to Galafold.[6] A person's Fabry disease mutation (genetic sequence) is typically identified and recorded when they are first diagnosed.[6]

The exact incidence of Fabry disease in Australia is hard to determine, due to the limited number of clinical studies in this area.[7] However, it is thought to be comparable to the rates of other western countries around the world, affecting between 1 in 40,000 to 1 in 117,000 live births.[2]

“Fabry Australia is delighted with the Australian Government’s decision to list Galafold on the LSDP,” said Megan Fookes OAM, Managing Director of Fabry Australia.

“Treatment for Fabry disease is lifelong. As the daughter of someone who passed away from the devastating impacts of Fabry disease, as a mother of a child with Fabry disease and as someone who lives with the condition myself - along with dear friends and colleagues - the importance of patients having access to the latest treatment developments as they become available cannot be underestimated.

“Today’s listing announcement means the Australian Fabry patient community with amenable mutations now have treatment choice when speaking to their doctors about how to best manage the debilitating effects of their disease.” said Ms Fookes.

Recognising the importance of Australian patients with Fabry and other rare disease patients, Amicus is simultaneously announcing the launch of its Australian commercial operations, based in Sydney. This follows the company initially setting up as a legal entity in Australia in September 2017.

“Amicus is very proud to be launching Australian operations in Sydney,” said Belinda (Blue) Kemball, General Manager of Asia-Pacific for Amicus Therapeutics Pty Ltd.

“Amicus is a global biotechnology company that is dedicated to developing innovative therapies for rare and orphan diseases, and today’s listing of Galafold on the LSDP for eligible Fabry disease patients is testament to our commitment to bring treatments to those patients who need them most. Amicus would like to thank the Australian Government and the Commonwealth Department of Health for making Galafold available on the LSDP. In addition, we recognise the tireless work of the Australian Fabry disease community – including clinicians, Fabry Australia, patients and their families – in advocating for access to new treatment options for this rare and debilitating disease.” said Mrs Kemball.

Galafold represents a clinically significant advance in treating Fabry disease in Australia, as the first new therapy to be approved for the rare condition in over 12 years.[1] It is expected that 35-50 per cent of diagnosed Fabry disease patients will be eligible for treatment with Galafold.[6]

Australian Galafold (migalastat) Important Safety Information[3]
Galafold is available as an oral tablet (123mg migalastat hard capsule, equivalent to 150mg migalastat hydrochloride hard capsules) and should be taken every other day.

INDICATION: Galafold is indicated for long-term treatment of adult and adolescent patients 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation.

CONTRAINDICATIONS: Galafold should not be given to patients who have a known hypersensitivity (allergy) to migalastat or any of its ingredients.

PRECAUTIONS: Galafold should not be used in patients with severe renal insufficiency or as an accompaniment with enzyme replacement therapy. Galafold should not be used in women of childbearing potential who are not using contraception, or in patients under the age of 16 years.

INTERACTIONS: Galafold currently has no known interactions with other medicines.

ADVERSE EFFECTS: The most common adverse reaction reported in clinical trials of Galafold was headache, which was experienced by 10 per cent of patients who received Galafold. Other common adverse events recorded included diarrhea, nausea, dizziness, numbness or burning feeling in the extremities (paraesthesia), muscle spasms, rash, fatigue, vertigo, abdominal pain, constipation, dry mouth, palpitations, defaecation urgency, indigestion (dyspepsia), pain, myalgia, pain in extremity, wry neck (torticollis), reduced sense of touch (hypoaesthesia), depression, high levels of protein in the urine (proteinuria), difficult or labored breathing (dyspnea), nose bleeds (epistaxis) and severe itching of the skin (pruritus).

Before taking Galafold® please review the Consumer Medicines Information available at https://www.tga.gov.au/artg

PBS Information: This product is not listed on the PBS.

Associate Professor Nicholls has been an investigator in clinical trials sponsored by Amicus. In relation to this Amicus media announcement, no compensation was provided to her, and the opinions expressed are her own.

~ ENDS ~

This media release has been distributed by opr health on behalf of Amicus Therapeutics Pty Ltd.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, USA and Japan, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is AT-GAA, a novel, late-stage, treatment for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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