



**Arix Bioscience plc**

**Imara presents initial Phase 2a data for IMR-687**

**LONDON, 18 June 2019:** Arix Bioscience plc (LSE:ARIX) ("Arix"), a global venture capital company focused on investing and building breakthrough biotech companies, notes that its portfolio company Imara, Inc. ("Imara") has presented positive initial data from its ongoing Phase 2a clinical trial evaluating the safety and efficacy of IMR-687, its lead programme for the treatment of Sickle Cell Disease, at the 24th Congress of the European Hematology Association (EHA) in Amsterdam.

The announcement can be accessed on Imara's website <http://imaratx.com/who-we-are/news-press-releases/> and the full text of the announcement from Imara is contained below.

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**About Arix Bioscience plc**

Arix Bioscience plc is a global venture capital company focused on investing in and building breakthrough biotech companies around cutting edge advances in life sciences.

We collaborate with exceptional entrepreneurs and provide capital, expertise and global networks to help accelerate their ideas into important new treatments for patients. As a listed company, we are able to bring this exciting growth phase of our industry to a broader range of investors.

Arix Bioscience plc is listed on the Main Market of the London Stock Exchange. For further information, please visit [www.arixbioscience.com](http://www.arixbioscience.com)

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**Imara Presented Positive, Interim Phase 2a Data on IMR-687 for the Treatment of Sickle Cell Disease at the 24<sup>th</sup> Congress of the European Hematology Association**

*Company encouraged by interim 13-week data in trial designed to assess the safety and tolerability of IMR-687 and explore the potential efficacy of IMR-687 on clinical outcome measures relevant to sickle cell disease*

**Cambridge, Mass., June 18, 2019** – Imara Inc., a clinical-stage biopharmaceutical company developing novel therapies for people living with sickle cell disease and other serious, inherited blood disorders, today announced promising interim, 13-week Phase 2a data on IMR-687 that were presented at the 24<sup>th</sup> Congress of the European Hematology Association (EHA) in Amsterdam. IMR-687 is an investigational, orally administered, highly potent and selective phosphodiesterase 9 (PDE9) inhibitor currently being evaluated as a potential disease-modifying therapeutic for sickle cell disease (SCD). Data presented at EHA demonstrated that treatment with IMR-687 in adult patients was generally well tolerated. The data also support the dual mechanism of action of IMR-687, with activity seen across both red and white blood cell biomarkers.

“These initial Phase 2a data demonstrate the potential of IMR-687 to significantly impact key biomarkers associated with the pathology of this serious disease,” said Biree Andemariam, M.D., Associate Professor at UConn School of Medicine and Director of the New England Sickle Cell Institute at UConn Health, and lead investigator for the trial. “In the laboratory we saw that IMR-687 inhibition of PDE9 increases intracellular cGMP levels, increases fetal hemoglobin expression, reduces sickling and hemolysis of red blood cells, and does not induce neutropenia. The interim Phase 2a data reflect trends that could be indicative of meaningful clinical translation of these important measures in SCD.”

The Phase 2a clinical trial is designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of escalating doses of IMR-687 administered once daily for 16 to 24 weeks in two groups of patients with SCD: those not on hydroxyurea (HU) (Population A) and those receiving a stable dose of HU according to the current standard of care (Population B). Blinded white cell markers in Population A (n=19), along with red cell markers and quality of life measures (ASCQ-Me<sup>®</sup>) (n=13) were analyzed. The blinded interim analysis was conducted on the monotherapy IMR-687 (Population A) subgroup. At 5 weeks, in the 100 mg dose group of Population A, there was a trend to reduced soluble P selectin (sPsel), soluble vascular cell adhesion molecule-1 (sVCAM) and myeloperoxidase (MPO) compared to placebo. At 13 weeks, in the 100 mg dose group of Population A, there was an increase (110%) in the percent of F-cells, which are red blood cells that contain fetal hemoglobin (HbF) which often precede rises in total HbF. A corresponding decrease in absolute reticulocyte count and the percentage of reticulocytes, with a trend towards improved pain as measured by ASCQ-Me<sup>®</sup>, was also observed at 13 weeks in Population A.

Blinded safety data for 27 patients in the Phase 2a clinical trial demonstrated that treatment with IMR-687 was generally well tolerated, with no clinically significant changes in white blood cell counts and no evidence of neutropenia. There were no treatment-related serious adverse events.

“We are encouraged by this interim Phase 2a analysis that reinforces our belief in the potential of IMR-687 as a single oral, once-a-day therapeutic,” said Rahul D. Ballal, Chief Executive Officer of Imara. “IMR-687 uniquely targets both red cell and white cell aspects of the disease, and we are working to expeditiously advance this novel therapy through clinical development, with a goal of delivering it to patients with SCD who are in need of innovative treatment options.”

Imara expects to announce additional interim Phase 2a data in the second half of 2019. Imara anticipates that these data will include safety and potential activity in additional patients, including patients in Population A that received higher doses and/or longer treatment duration with IMR-687 as well as the first data on patients in Population B.

### **IMR-687 Phase 2a Clinical Trial Design**

IMR-SCD-102 is a Phase 2a, randomized, double-blind, placebo-controlled study of IMR-687. It is designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of escalating doses of IMR-687 administered once daily for 16 to 24 weeks in two groups of patients with SCD: those not on hydroxyurea (HU) (Population A) and those receiving a stable dose of HU according to the current standard of care (Population B). Participants in Population A receive either IMR-687 or placebo for a total of 24 weeks. On Day 1, subjects are randomized in a 1:1:1 ratio to receive oral IMR-687 50 mg, IMR-687 100 mg or placebo daily for the first 12 weeks. Each participant’s dose is doubled at week 13 following the review of clinical safety data. Participants in Population B are randomized in a 2:1 ration and receive either a 50 mg dose of IMR-687 or placebo, with dose escalation to 100 mg (or placebo) after four weeks and review of the clinical safety data. The Adult Sickle Cell Quality of Life Measurement Information System (ASCQ-Me<sup>®</sup>), is also administered at baseline, 13 weeks and at the end of the study.

### **About IMR-687**

IMR-687 has been designed to address the underlying pathology of sickle cell disease. An orally administered, highly potent and selective phosphodiesterase 9 (PDE9) inhibitor, IMR-687 has the potential to be a disease-modifying therapeutic for sickle cell disease as well as other hemoglobinopathies. Pre-clinical data demonstrate IMR-687 reduces both the sickling of red blood cells and blood vessel occlusion that cause debilitating pain, organ damage, and early mortality in affected patients. In a Phase 1 clinical trial in healthy volunteers, IMR-687 was demonstrated to be well-tolerated. IMR-687 is currently in a Phase 2a clinical trial in patients with sickle cell disease. IMR-687 has been granted U.S. Orphan Drug Designation, U.S. Rare Pediatric Designation and Fast Track Designation by the Food and Drug Administration (FDA).

### **About Sickle Cell Disease**

Sickle cell disease is a rare, genetically inherited condition that alters hemoglobin, the protein in red blood cells that transports oxygen throughout the body. The altered hemoglobin distorts red blood cells into a sickle, or crescent, shape. Painful episodes can occur when sickled red blood cells, which are stiff and inflexible, get stuck in small blood vessels. These episodes deprive tissues and organs of oxygen-rich blood and can lead to vaso-occlusive crisis (VOC), acute chest syndrome (ACS), and permanent damage to organs including the liver, spleen, kidney and brain.

Sickle cell disease represents a critical unmet medical need globally, as a rare disease in many parts of the world including in the United States and as an endemic condition in certain African countries.

**About Imara**

Imara Inc. is committed to transforming the lives of people with sickle cell disease and other serious hemoglobinopathies, including beta thalassemia, by developing oral small molecule therapeutics that are designed to be easy to administer and use across the world. Imara is currently advancing IMR-687, a highly selective, potent small molecule inhibitor of PDE9 with a dual mechanism of action targeting both red and white blood cells, in an ongoing Phase 2a clinical trial in patients with sickle cell disease. In March 2019, Imara closed a \$63M Series B with leading life science investors New Enterprise Associates, OrbiMed Advisors, Arix Bioscience plc, RA Capital, Rock Springs Capital, Pfizer Venture Investments, Lundbeckfonden Ventures, Bay City Capital and Alexandria Venture Investments. For more information, please visit [www.imaratx.com](http://www.imaratx.com).

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