Shire, Microsoft and EURORDIS form Global Commission to accelerate time to diagnosis for children with rare diseases

Alliance aims to shorten the often multi-year journey that patients and families endure before being diagnosed with a rare disease

On average, it takes 5 years before a rare disease patient, of which approximately half are children, receives the correct diagnosis

Cambridge, Ma., Redmond, Wash., and Brussels, Belgium – February 20, 2018 – Shire plc (LSE: SHP, NASDAQ: SHPG), Microsoft and EURORDIS-Rare Diseases Europe today announced a strategic alliance to address the diagnostic challenge for patients living with a rare disease. The long road to diagnosis is one of the most important issues affecting the health, longevity and well-being for rare disease patients and their families.

The Global Commission to End the Diagnostic Odyssey for Children ("the Global Commission") is a multi-disciplinary group of experts with the creativity, technological expertise and commitment required to make a major difference in the lives of millions of children and their families. The Global Commission will develop an actionable roadmap to help the rare disease field to shorten the multi-year diagnostic journey, considered a key to a longer, healthier life.

Within its roadmap, the Global Commission will offer recommendations designed to address core barriers preventing timely diagnosis impacting all rare disease patients, of which approximately half are children[1], such as:

- Improving physicians' ability to identify and diagnose patients with a rare disease in order to begin care and treatment
- Empowering patients and their families to have a more active role in their health care
- Providing high-level policy guidance to help achieve better health outcomes for rare disease patients

Under the leadership of its co-chairs, Flemming Ornskov, M.D., M.P.H., Chief Executive Officer, Shire, Simon Kos, M.D., Chief Medical Officer and Senior Director, Worldwide Health, Microsoft, and Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe, the Global Commission will bring together a dedicated group of technology innovators, patient advocates, healthcare providers, researchers, family members and other experts from around the world to tackle one of the most serious and heartbreaking challenges within the rare disease space.

"As a physician with training in pediatrics, I've seen firsthand the devastating effect not having an accurate diagnosis can have on patients, their families, as well as on the health care providers working to help them. Accelerating the time to diagnosis is critical to improving outcomes for patients and health systems," said Ornskov. "This Global Commission is passionate about bringing forward new and personalized solutions in diagnostics and I'm confident our work will help to transform the lives of children living with a rare disease."

There are more than 6,000 identified rare diseases and it is estimated that rare diseases affect 300 to 350 million people worldwide.[2] Many patients endure lifelong suffering and about half of all rare diseases begin in childhood.

"We have an opportunity to harness the power of technology to tackle this painful issue that has affected so many. We're seeking innovative ways to integrate emerging technologies into our efforts, which will play a critical role as we strive to impact the diagnosis journey," said Kos. "Microsoft is committed to this mission and I believe the Global Commission's wide range of expertise, along with the infusion of technology, will change the state of rare disease diagnosis."

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[1] Global Genes. RARE Disease: Facts and Statistics. https://globalgenes.org/rare-diseases-facts-statistics/.
[2] European Medicines Agency. Orphan Medicines in the EU. http://www.ema.europa.eu/docs/en_GB/document_library/Leaflet/2017/12/WC500240710.pdf.
*Press release updated March 3, 2018 due to additional member added to the Global Commission

The Global Commission is beginning its work in early 2018 and expects in early 2019 to publish a roadmap that encapsulates the findings of its work together. Over the course of 2018, the Global Commission will gather input from patients, their families, and other expert advisors to gain additional key insights and help drive solutions to speed the rare disease diagnosis timeline.

“Today, many children around the world are living with a rare disease that remains either undiagnosed or misdiagnosed. This can delay proper care and treatment and cause isolation, discrimination, social exclusion, and also contributes to a waste in human resources” said Le Cam. “We are excited to begin collaborating with such a distinguished and diverse group of experts and believe together we can facilitate and accelerate time to diagnosis helping families around the world.”

About the Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease
The Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease was created in collaboration by Shire, Microsoft and EURORDIS-Rare Diseases Europe. The purpose of the Global Commission is to establish a roadmap for the rare disease field that focuses on solutions to core barriers preventing timely diagnosis for all rare diseases – with an emphasis on those affecting children. The Global Commission brings together representatives from multiple sectors to provide diverse perspectives on rare disease diagnostics.

The Global Commission Members**:**

Flemming Ornskov, M.D., M.P.H., CEO, Shire (Co-Chair)
Simon Kos, M.D., Chief Medical Officer and Senior Director, Microsoft Worldwide Health (Co-Chair)
Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe (Co-Chair)
Moeen Al-Sayed, M.D., Chairman of Medical Genetics, King Faisal Specialist Hospital
Kym Boycott, M.D., Ph.D., FRCPC, FCCMG, Clinical Geneticist, Children’s Hospital of Eastern Ontario; Senior Scientist, CHEO Research Institute; and Professor, Department of Pediatrics, University of Ottawa
Roberto Giugliani, M.D., Ph.D., Medical Genetics Service, Hospital de Clinicas de Porto Alegre, and Professor of Medical Genetics, Federal University of Rio Grande do Sul, Brazil
Kevin Huang, President, Chinese Organization for Rare Disorders
Derralynn Hughes, Ph.D., Clinical Director Haematology Oncology and Palliative care, Senior Lecturer and Investigator Lysosomal Storage Disorders Unit, Royal Free & University College Medical School
Daniel MacArthur, Ph.D., Institute Member, Co-Director of the Medical and Population Genetics Program, Broad Institute
Maryam Matar, M.D., Founder and Executive Director, UAE Genetic Diseases Association
Dau-Ming Niu, M.D., Ph.D., Director, Center for Medical Genetics, Taipei Veterans General Hospital
Mike Porath, Founder and CEO, The Mighty
Arndt Rolfs, M.D., CEO, Centogene
Richard Scott, Ph.D. Clinical Lead for Rare Disease, 100,000 Genomes Project at Genomics England and Consultant and Honorary Senior Lecturer in Clinical Genetics, Great Ormond Street Hospital for Children and the UCL Institute of Child Health
Marshall Summar, M.D., Director, Rare Disease Institute & Division Chief, Genetics and Metabolism, Children’s National
Durhane Wong-Rieger, Ph.D., President & CEO, the Canadian Organization for Rare Disorders and Council Member, Rare Diseases International

*Press release updated March 3, 2018 due to additional member added to the Global Commission

About Shire
Shire is the global leader in serving patients with rare diseases. We strive to develop best-in-class therapies across a core of rare disease areas including hematology, immunology, genetic diseases, neuroscience, and internal medicine with growing therapeutic areas in ophthalmics and oncology. Our diversified capabilities enable us to reach patients in more than 100 countries who are struggling to live their lives to the fullest.

We feel a strong sense of urgency to address unmet medical needs and work tirelessly to improve people's lives with medicines that have a meaningful impact on patients and all who support them on their journey.

www.shire.com

About Microsoft
Microsoft (Nasdaq "MSFT" @microsoft) is the leading platform and productivity company for the mobile-first, cloud-first world, and its mission is to empower every person and every organization on the planet to achieve more.

About EURORDIS-Rare Diseases Europe
EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 700 rare disease patient organisations from more than 60 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit www.eurordis.org.

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