

IFPMA launches new policy principles and report on Rare Disease Day 2017 to benefit patients, healthcare and society

- New [set of policy principles](#) outlines critical elements for a global framework on rare disease policy.
- New [report](#) “Rare diseases: shaping a future with no one left behind” is a new resource to understand key challenges in areas such as R&D, clinical trials, diagnosis, and access to treatment.
- More than 560 medicines are currently being developed for patients with rare diseases thanks to new technologies and a growing scientific understanding of these diseases¹.

Geneva, 28 February – Celebrating Rare Disease Day 2017, IFPMA launches today new resources to help increase understanding and awareness of rare diseases. These include a [set of policy principles](#) outlining critical elements for a global framework on rare disease policy and a [report](#) that describes key challenges in areas such as R&D, clinical trials, diagnosis, and access to treatment.

Rare diseases pose unique challenges to patients, their families, society, healthcare professionals and healthcare systems. There are between 5,000-8,000 rare diseases, and new rare diseases are being discovered all the time. They affect 350-400 million people, representing 6-10% of the world population.

While significant focus has been given lately to rare diseases in the global policy agenda, many countries still lack tailored policy frameworks to help address a large unmet medical need. Most rare diseases are genetic and can be associated with life-long disability, often starting in childhood. Many are chronic, degenerative and life-threatening conditions. It can take over five years to reach the right diagnosis, and many rare disease patients are undiagnosed.

IFPMA’s vision in rare diseases is to promote equitable and timely access to the tools, appropriate healthcare infrastructure and supportive care that patients need to manage their diseases.

“Our Members believe that each person with a rare disease should expect to be treated with dignity and appropriate care” – said Thomas Cueni, Director General, IFPMA. “This set of principles we release today will help us contribute towards discussions on a global policy ecosystem that recognizes the need to improve the lives of patients with rare diseases”.

IFPMA’s rare diseases principles are based on industry’s shared experiences. A supportive policy environment for rare diseases is one that helps to increase understanding of these diseases and the way they affect patients, to stimulate more research, to encourage appropriate disease management and to empower patients and their wider communities. Additionally, political awareness is essential to create supportive policies for rare disease patients, such as legislation on equal access to healthcare, orphan drugs, or employment and education for individuals with disabilities.

IFPMA calls for multi-stakeholder collaboration and dialogue to ensure patients’ access to treatments. This approach is even more critical as the different nature and maturity of national healthcare systems across the world calls for solutions which are tailored to local needs.

“This work is a key contribution to Rare Disease Day 2017 and an invite to our partners for continuous dialogue and action to commit to solutions that may control and ultimately cure patients with rare diseases”, said Kevin Loth, Chair of the IFPMA Rare Diseases Working Group and Vice President of Corporate Affairs and Policy, Celgene.

¹ Medicines in Development for Rare diseases, ALS Association and PhRMA. [See link.](#)



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About IFPMA

news release

IFPMA represents research-based pharmaceutical companies and associations across the globe. The research-based pharmaceutical industry's 2 million employees research, develop and provide medicines and vaccines that improve the life of patients worldwide. Based in Geneva, IFPMA has official relations with the United Nations and contributes industry expertise to help the global health community find solutions that improve global health. IFPMA advocates policies and practices that encourage the discovery of and access to life-saving and life-enhancing medicines and vaccines, for people everywhere.

For further information, please contact

Morgane De Pol
Manager, Communications & Public Affairs
Email: m.depol@ifpma.org
Tel: +41 22 338 32 20