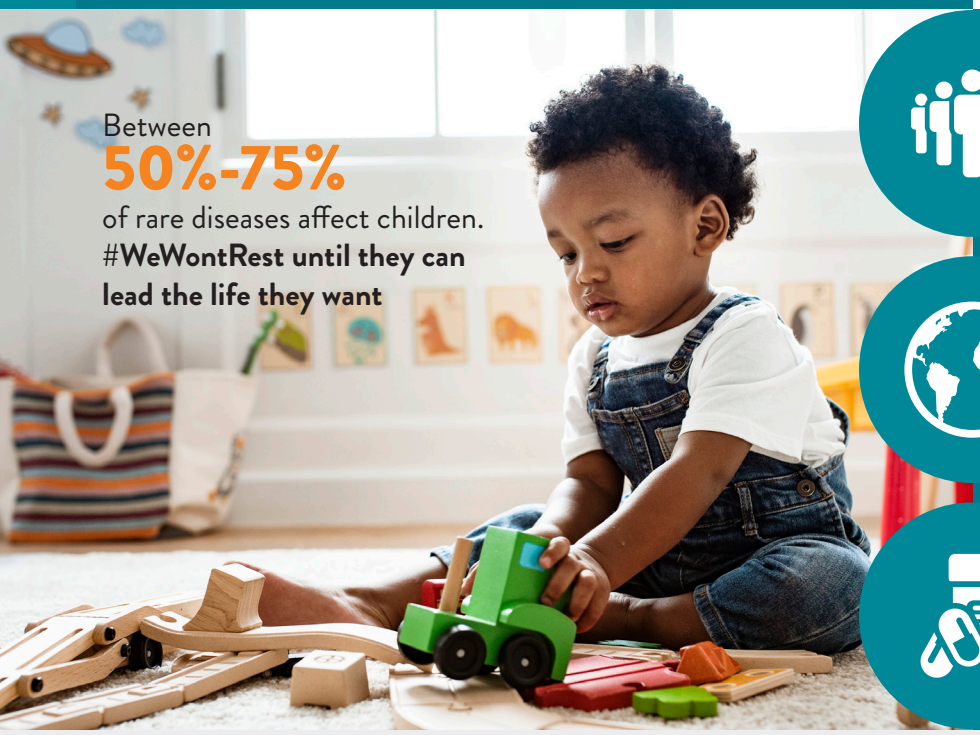
#WEWONTREST UNTIL WE MAKE TREATMENTS FOR RARE DISEASE LESS RARE

The pharmaceutical industry is working tirelessly to find new treatments for the **1 in 17**[1] European patients living with rare diseases.

efpia
European Federation of Pharmaceutical Industries and Associations



Between **50%-75%** of rare diseases affect children. **#WeWontRest until they can lead the life they want**

A disease or disorder is defined as rare in Europe when it affects no more than **1 in 2000**[1]

Between **5000-8000**[2] distinct rare diseases exist globally

Only **~5%** of rare diseases are estimated to have an approved treatment[3]

WHEN POLICY DRIVES PROGRESS

The EU Orphan Medicines Regulation[4] provides protocol assistance, reduced fees for regulatory activities, additional incentives for SMEs, and 10 years of market exclusivity for approved OMPs. The regulation creates an environment that fosters investment and innovation in Europe, leading to a greater understanding of rare diseases and importantly to new treatments.

Rare diseases affect around **30 million people**[1] in Europe.

Through continued research and innovation, **#WeWontRest until treatments for rare conditions become less rare.**

Since the EU Orphan Regulation was adopted in 2000:

The number of orphan medicines went from only 8 products to more than **160 today**[5].

2,121 orphan designations[5] between 2000-2018.

Clinical trials in rare diseases **increased by 88%**[6] between 2006-2016.

24 European Reference Networks[7] have been established.

by 2018, **23 European countries**[8] had developed national rare disease plans.

220 SMEs were created, responsible for the development of **51% of orphan medicines** in Europe.

THE POTENTIAL OF A TREATMENT CAN ONLY BE REALISED IF PATIENTS HAVE ACCESS TO IT

For every new treatment developed, there are many more rare diseases still without one.

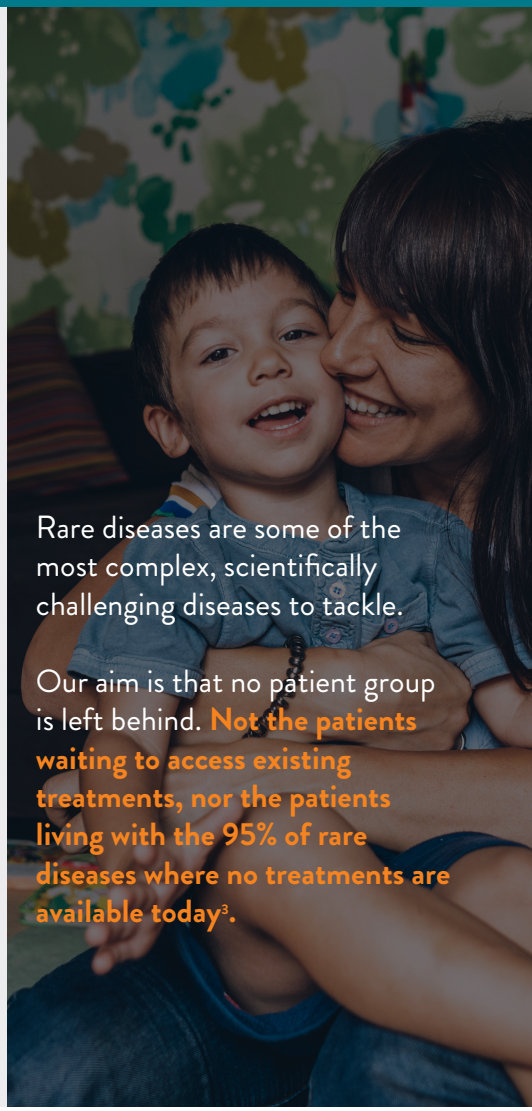
Addressing the needs of patients waiting to access existing treatment and the patients living with the 95% of rare diseases[3] where no treatments are available today means standing by the Orphan Regulation. Maintaining Europe's world class regulatory and incentives system will continue to support further research into the next generation of treatments.

Managing the introduction of new treatments is challenging for healthcare systems, particularly in the case of cell and gene therapies which can replace a life-time of care with a one-time treatment but where the costs are usually borne by the health system upfront.

Industry, healthcare systems and governments share the goal and responsibility of ensuring patient access to the new treatments of today and tomorrow. There has been significant progress:

A number of countries across Europe have adapted HTA and reimbursement policies to best capture the value that these products bring.

Companies already engage in innovative arrangements, such as managed entry agreements and early access schemes, to support patients' access to new medicines.



Rare diseases are some of the most complex, scientifically challenging diseases to tackle.

Our aim is that no patient group is left behind. **Not the patients waiting to access existing treatments, nor the patients living with the 95% of rare diseases where no treatments are available today[3].**

SO MUCH MORE NEEDS TO BE DONE

We believe it is time for a different type of conversation. **EFPIA supports the EU Health Coalition's call**[9] for a multi-stakeholder **High Level Forum** for better access to health innovation to bring all actors together to discuss how to ensure access to new treatments and technologies today, medical innovation for tomorrow and sustainable healthcare systems in a globally competitive Europe.

1 EURORDIS. Available at: http://www.eurordis.org/about-rare-diseases
2 https://ec.europa.eu/health/non_communicable_diseases/rare_diseases_en
3 Global Genes, Rare diseases: Facts and Statistics, https://globalgenes.org/rare-diseases-facts-statistics/
4 Regulation 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2000:018:0001:0005:en:PDF
5 EMA. Available at: https://www.ema.europa.eu/documents/other/orphan-medicines-figures-2000-2018_en.pdf
6 Evaluating the Orphan Regulation and its impact on patients and rare disease R&D in the European Union. Available at: http://www.pugatch-consilium.com/reports/Benchmarking_success.pdf
7 European Commission. Available at: https://ec.europa.eu/health/sites/health/files/ern/docs/2017_brochure_en.pdf
8 http://www.europlanproject.eu/NationalPlans?idMap=1
9 EU Health Coalition. Available at: https://euhealthcoalition.eu/