#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.

AiFD
Araştırmacı İlaç Firmaları Derneği
Association of Research-Based Pharmaceutical Companies

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]

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 164 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.

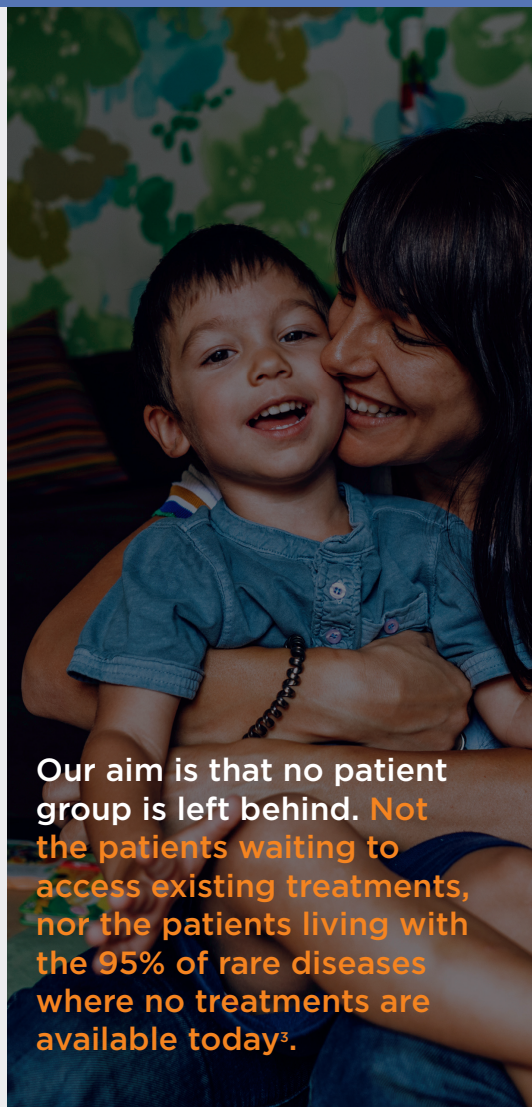
THE POTENTIAL OF A TREATMENT CAN ONLY BE REALISED IF PATIENTS HAVE ACCESS TO IT

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.

With the introduction of a regulatory and incentive system under global standards, more research on next-generation treatments will be supported.

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

According to the results of a survey conducted by AIFD among its members in 2018, more than 300 patients from Turkey participated in more than 30 clinical studies conducted by 10 multinational pharmaceutical companies in the field of rare diseases[9]. According to the results of the survey conducted in 2019, 21 clinical studies are being conducted in Turkey for orphans drugs, which are currently do not have access[10].

We believe that it is time for us to say different things. In order to take the most important concrete steps in this field, all stakeholders must meet on common grounds and agree on a common value system, and then produce content accordingly. In this context, AIFD positions itself as a stakeholder that facilitates interaction and plays a scientifically supportive role among all stakeholders.

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 AIFD Orphan Drug Survey 2018
10 AIFD Orphan Drug Survey 2019