RARE DISEASES AS A NATIONAL PRIORITY

Designing and implementing national policies, strategies, and programs with the aim of contributing to the well-being and fulfilling the rights of all people living with a rare disease in Greece.

Michael Himonas, SfEE General Manager






March 2, 2022

Pharma Industry is working tirelessly to find new treatments for the patients living with rare diseases

In 2000 only 8 orphan-like products were available; In 20 years this number has risen to more than 190 today

Life-changing progress in treating rare diseases over the last two decades

Research and development into rare diseases is right at the cutting edge of science and technology

This incredible process has been facilitated by the EU Orphan Medicines Regulation

Sources: www.efpia.eu; www.eurordis.org/about-rare-diseases; 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

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The number of clinical trials on rare diseases has increased by 88% between 2006 and 2016

577 rare disease products use new technologies

(such as cell and gene therapies, antisense RNA interference therapy and monoclonal antibodies to precisely target the disease site)

A survey of 180 expert physicians shows that of the 26 medicines identified, 10 were first developed for rare diseases

2,121 new orphan designations leading to 164 authorised new treatments for around 90 rare diseases between 2000 and 2018

Sources: www.efpia.eu; www.eurordis.org/about-rare-diseases; 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

Orphan and Paediatric Regulations: two European success stories

1

The Orphan and Paediatric Regulations have been two European success stories, and the European legislator, the EMA and the pharmaceutical industry can be proud of the results achieved for patients in Europe since their introduction

2

More is needed to address the needs of patients – building on what has already been achieved thanks to the incentives and rewards embedded in the Regulations, and the investment and scientific progress achieved by research in these fields

3

We share EC's objectives to foster further biopharmaceutical development for children and patients suffering from rare disease, and to ensure faster and better accessibility of medicines across Member States.

4

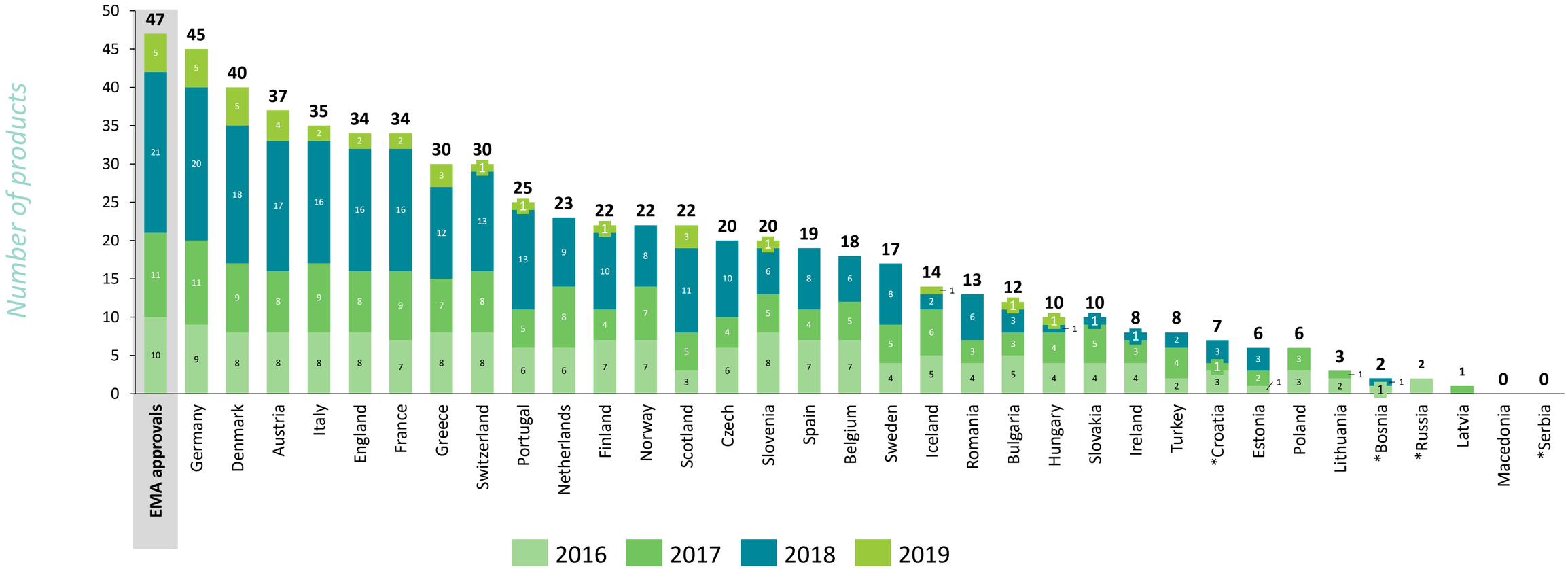
From just 8 orphan-like medicines authorised before the adoption of the Orphan Regulation in 2000, a further 190 initial orphan marketing authorisations and 34 extensions of indication in 133 disease areas have since been granted by the EMA, addressing the needs of up to 6.3 million rare disease patients

5

Since 2007, the Paediatric Regulation has resulted in over 400 new treatment options for children (new marketing authorisations and new indications); thereof 159 have been authorised since 2017.

Orphan availability by approval year (2016 - 2019)

The **total of availability by approval year** is the number of medicines available to patients in European countries (for most countries this is the point at which the product gains access to the reimbursement list†), split by the year the product received marketing authorization in Europe.



European Union average: 19 products available (41%) (excludes data from Cyprus, Malta, and Luxembourg as these countries are not included in the study) †In most countries availability equates to granting of access to the reimbursement list, except in DK, FI, NO, SE some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative.

Orphan rate of availability (2016 - 2019)

The rate of availability, measured by the number of medicines available to patients in European countries as of 2020. For most countries this is the point at which the product gains access to the reimbursement list†, including products with limited availability.

European Union average: 19 products available (41%) (excludes data from Cyprus, Malta, and Luxembourg as these countries are not included in the study) †In most countries availability equates to granting of access to the reimbursement list, except in DK, FI, NO, SE some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative.

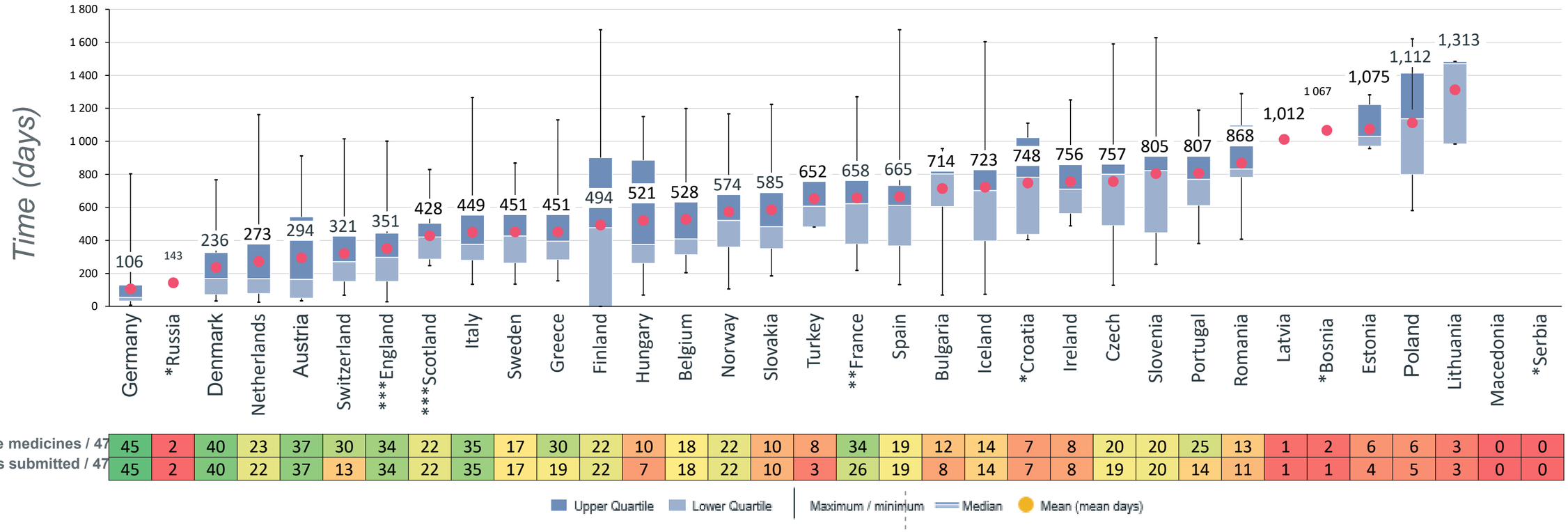
Orphan rate of availability (%, 2016 – 2019)

The **rate of availability** is the number of medicines available to patients in European countries (for most countries this is the point at which the product gains access to the reimbursement list†). This includes all medicines status to provide a complete picture of the availability of the cohort of medicines studied.

European Union average: 19 products (41%) (excludes data from Cyprus, Malta, and Luxembourg as these countries are not included in the study); EU averages: 12% of the study cohort is subject to limited availability; 37% of available products have limited availability across EU countries. Ireland and Norway did not submit information on restrictions to available medicines meaning LA* is not captured in these countries. As Ireland has been included in the EU average calculations, the EU average number of products with limited availability may be understated. †In most countries availability equates to granting of access to the reimbursement list, except in DK, FI, NO, SE some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative.

Orphan time to availability (2016 – 2019)

The **time to availability** (previously know as length of delay) is the days between EMA marketing authorisation and the date of availability to patients in European countries (for most this is the point at which products gain access to the reimbursement list†).



Available medicines / 47	45	2	40	23	37	30	34	22	35	17	30	22	10	18	22	10	8	34	19	12	14	7	8	20	20	25	13	1	2	6	6	3	0	0
Dates submitted / 47	45	2	40	22	37	13	34	22	35	17	19	22	7	18	22	10	3	26	19	8	14	7	8	19	20	14	11	1	1	4	5	3	0	0

European Union average: 653 days (mean) (excludes data from Cyprus, Malta, and Luxembourg as these countries are not included in the study) †In most countries availability equates to granting of access to the reimbursement list, except in DK, FI, NO, SE some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative.; **In France, some innovative products without competitors can be made available prior to market authorisation under the system of Temporary Authorisations. As these are not taken into account in the analysis, the average would be lower. ***In the UK, MHRA's Early Access to Medicines Scheme provides access prior to marketing authorisation but is not included within this analysis, and would reduce the overall days for a small subset of medicines.

Our commitment

Some diseases are rare. Treating them shouldn't be.
Multi-stakeholders' collaborations is needed.
#WeWontRest until we make treatments for rare disease less rare

https://www.youtube.com/watch?v=rphrZ0DonPs

Thank you!