



Rare Disease Day 2020

- 13th edition on Saturday 29 February 2020
- A rare day for rare diseases in this leap year!
- Key campaign message: **Rare is many.**
Rare is strong. Rare is proud!



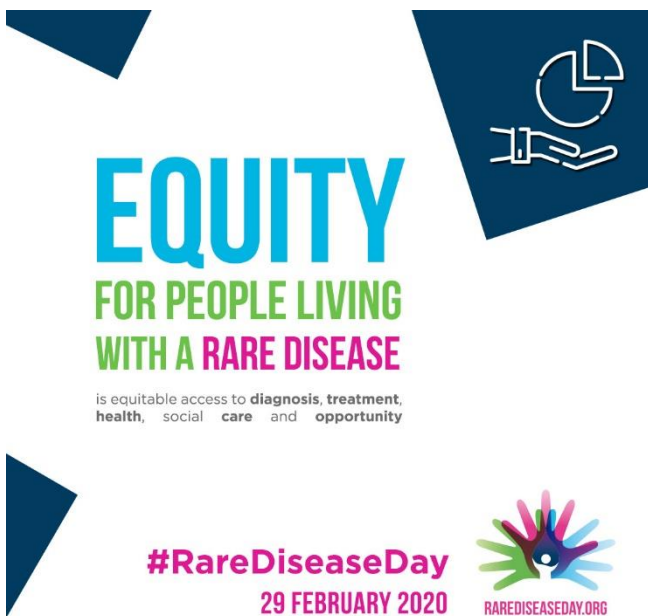
Campaign coordinated by EURORDIS and 60 national alliance partners representing 53 countries.



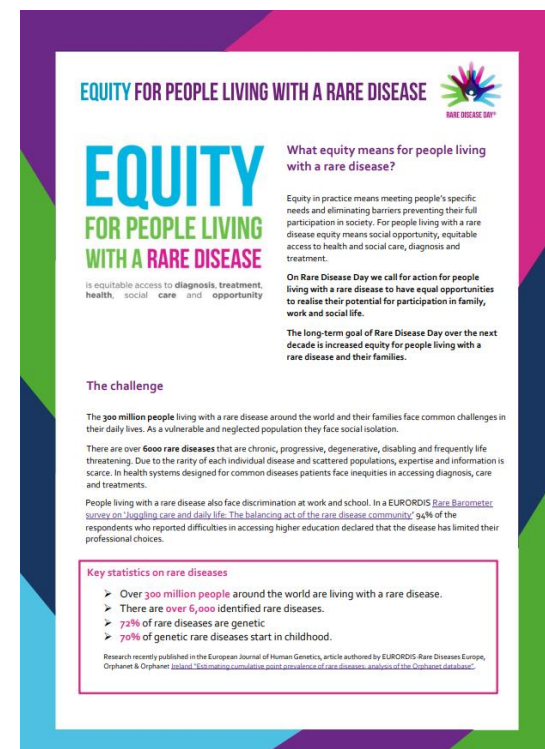
Policy communication

Calling for increased equity

- On Rare Disease Day we call for increased equity for people living with a rare disease and their families.
- New fact sheet released on 'Advocating for equity for people living with a rare disease'



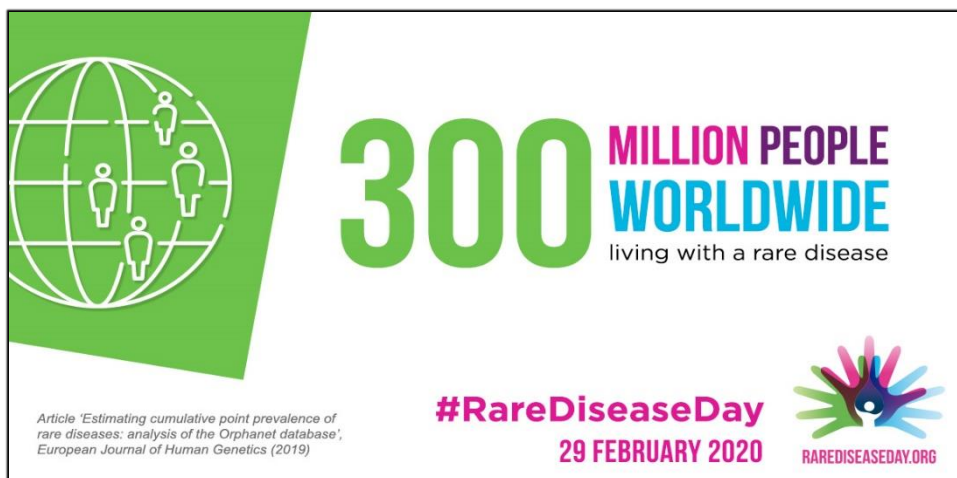
RAREDISEASEDAY.ORG



Rare Disease Day campaign materials

The Rare Disease Day campaign materials for print, online and social media were made editable and adaptable and available to download from rarediseaseday.org/downloads

The materials were translated into **over 30 languages!**



[RAREDISEASEDAY.ORG](https://rarediseaseday.org)





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ادر سنجد أنهم
تعداد السكاني لـ

أكبر
دولة
العالم

#RareDiseaseDay
rarediseaseday.org
#يوم_الأمراض_النادرة



RARE DISEASE

اير

DE LAS MAS DE
6,000+
ENFERMEDADES
RARAS
IDENTIFICADAS
EN MEXICO NO SABEMOS CON CUANTAS CONVIVIMOS
#REGISTRO NACIONAL

Article 'Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database',
European Journal of Human Genetics (2019)

#RareDiseaseDay



#RareDiseaseDay
rarediseaseday.org

SOMOS
300 MILLONES
EN EL MUNDO

MUESTRA TU APOYO ESTE DIA
29 FEBRERO 2020

Somos aproximadamente 3 millones de personas en México con una enfermedad rara.



#RareDiseaseDay
rarediseaseday.org

NOUS SOMMES
3 MILLIONS*

SHOW YOUR SUPPORT THIS RARE DISEASE DAY
29 FEBRUARY 2020

* 3 millions de personnes concernées par l'une des 7000 maladies rares

RARE DISEASE DAY®

@rarediseaseday @rarediseaseday @rarediseasedayofficial



#RareDiseaseDay
rarediseaseday.org

SELTEN IST
STARK



TAG DER S
29. FE

ΙΣΟΤΗΤΑ
ΓΙΑ ΤΑ ΑΤΟΜΑ ΠΟΥ ΖΟΥΝ
ΜΕ ΜΙΑ ΣΠΑΝΙΑ ΠΑΘΗΣΗ

είναι η δίκαιη πρόσβαση στη διάγνωση,
τη θεραπεία, την υγεία, τη φροντίδα
αλλά και τις κοινωνικές ευκαιρίες.

Nadir hastalıklar
halihazırda

DÜNYA
NÜFUSUNUN %5'İNİ
Etkiliyor

Kaynak: 'Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database' makalesi
- European Journal of Human Genetics (2019)



29 FEBBRAIO 2020

3 PIÙ GRANDE
DEL MONDO

#RareDiseaseDay
#GiornatadelleMalattieRare



RARE DISEASE DAY®



RARE DISEASE DAY®

Smart Vision School
DUBAI

27 FEBRUARY 2020



Thousands of events organised in over 100 countries + regions



New Countries in 2020

Kosovo



Shoqata e
Sëmundjeve
të Rralla

Gabon



Iraq



Pediatric Teaching
Hospital
Sulaymaniyah

Rwanda



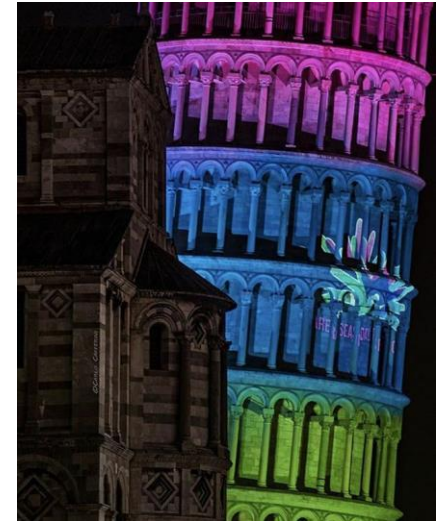
Monuments and buildings



Australia



Mexico



Pisa, Italy

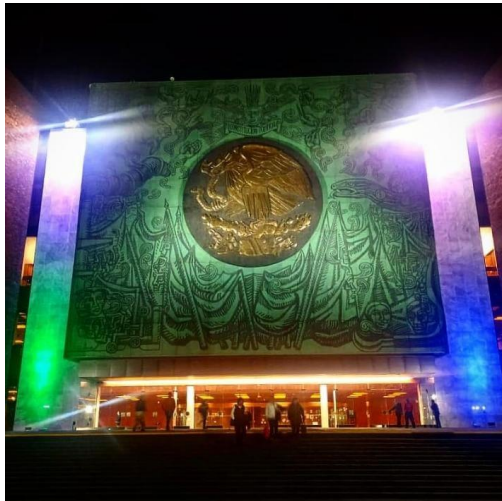


Rome, Italy

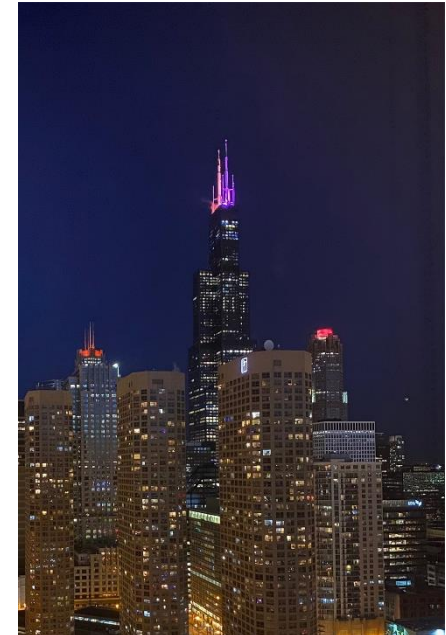
Monuments and buildings



Genova, Italy



Mexico



Chicago, USA



Murcia, Spain



Valencia, Spain



Peru

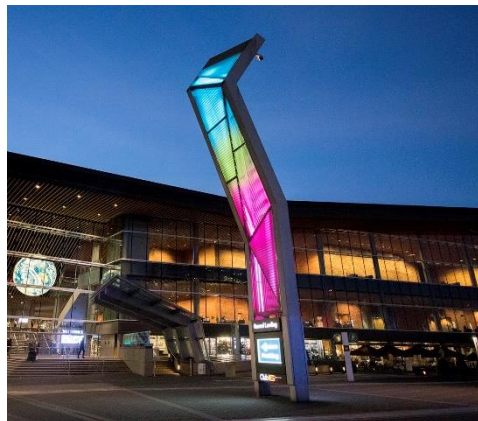
Monuments and buildings



New York City, USA



Peshawar, Pakistan



Vancouver, Canada



Vancouver, Canada

Rare Disease Day on social media!

#RareDiseaseDay trended in at least 13 countries.

Support from social media influencers



@j__vedder, 97,9K followers



@tummyandgummy, 132K followers



@sarageurts, 94,5K followers



@oddllyyyvie, 876K followers



Support from EU Health



 **EU_Health** 
@EU_Health

It's #RareDiseaseDay!

Have a listen to Jean-Yves Blay wish for the survival rates of rare cancer patients to be significantly improved, thanks to collaboration within the European Reference Networks (ERNs)

#EUCancerPlan

Visit ec.europa.eu >



European
Reference
Network

966 views 0:16 / 0:26

International media coverage

World Economic Forum



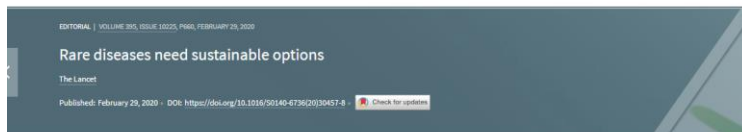
Global Health | Future of Health and Healthcare | Healthy Futures

It takes far too long for a rare disease to be diagnosed. Here's how that can change



The waiting game: a 'diagnostic odyssey' means patients can suffer for years before a diagnosis

THE LANCET



Article info

Figures

Feb 29 marks Rare Disease Day, an opportunity to reflect on the lives of the estimated 400 million people globally who are affected by one of 6000-8000 rare diseases, most of them genetic. These diseases were long neglected, but large international initiatives such as EURORDIS and Rare As One have been set up to represent patients' voices, highlight the disease burden (including psychological aspects) on patients and their families, share data and knowledge, promote collaboration, and fund research into the causes of rare diseases and novel therapeutics. As yet, targeted drugs are available for only a few hundred of these disorders, leaving millions of patients without any specific treatment options.

Legislation such as the Orphan Drug Act 1983 in the USA and Regulation 141/2000 in the EU incentivise biopharmaceutical companies to develop new drugs for rare diseases, with the prospect of fast-track designation and longer market exclusivity. Although this approach has resulted in hundreds of new orphan drugs, the small market of rare diseases is often cited as the reason for developers to set premium drug prices. Voretigene neparvovect—a gene therapy for RPE65-mutated Leber congenital amaurosis—was approved in 2017 by the US Food and Drug Administration and in 2018 by the European Medicines Agency, yet the €600 000 treatment cost is prohibitive to most patients. In the UK, NHS England has reached a NICE-endorsed deal with the drug manufacturer to provide this treatment as part of the NHS Long Term Plan, and last week the first patients were treated at two hospitals in London.

This agreement is good news because until now no treatment was available, and an estimated 100 people with Leber congenital amaurosis in the UK will have access to this gene therapy for the first time. But such a costly approach to treatment provision is not sustainable for most rare diseases. The best hope is that novel therapies developed using funds from non-commercial organisations, which can prioritise patients rather than profit, will provide the treatments these patients need.



Forbes

Forbes

1,618 views | Feb 28, 2020, 05:32am

The Challenges Of Combating Rare Diseases – And Five Innovations Making A Real Difference



Claire Novorol Contributor @

Healthcare

I am co-founder of Ada Health, writing about healthcare and AI.

February 29, 2020 is [Rare Disease Day](#), an annual event that aims to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives.

I've seen first-hand the difficulties that patients with rare diseases can face. In my first week as an NHS geneticist, I met a child who, despite seeing highly specialised doctors for over a year, had not yet been diagnosed because their symptoms, findings and lab tests matched no known disease. It was only after trawling medical databases and case studies that I was able to find the likely diagnosis, subsequently confirmed. The disease was so rare that naming it here could identify the patient.

Rare Disease Day, established in 2008 by the [European Organisation for Rare Diseases \(EURORDIS\)](#), has helped to amplify the great work being done by patient associations, which are supported by international and national organisations such as EURORDIS and NORD, to bring together

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The Lancet

