Integración del paciente en la investigación y ensayos clínicos

Julián Isla Gómez

Federación Europea de Síndrome de Dravet
Fundación 29

SOCIETAT CATALANA DE FARMÀCIA CLÍNICA

29 foundation twenty-nine

Microsoft Consulting Services. AI

Presidente y fundador

Director Científico

TAG. Therapeutic Advisory Group

Miembro del COMP

Comité científico asesor


ALFREDO LANDA
¡VENTE A ALEMANIA, PEPE!
TINA SAINZ • JOSE SACRISTAN
ANTONIO FERRANDIS • GEMMA CUERVO • FERNANDO GUILLEN
DIRIGIDA POR PEDRO LAZAGA
VICENTE ESCRIVA Y VICENTE COELLO

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

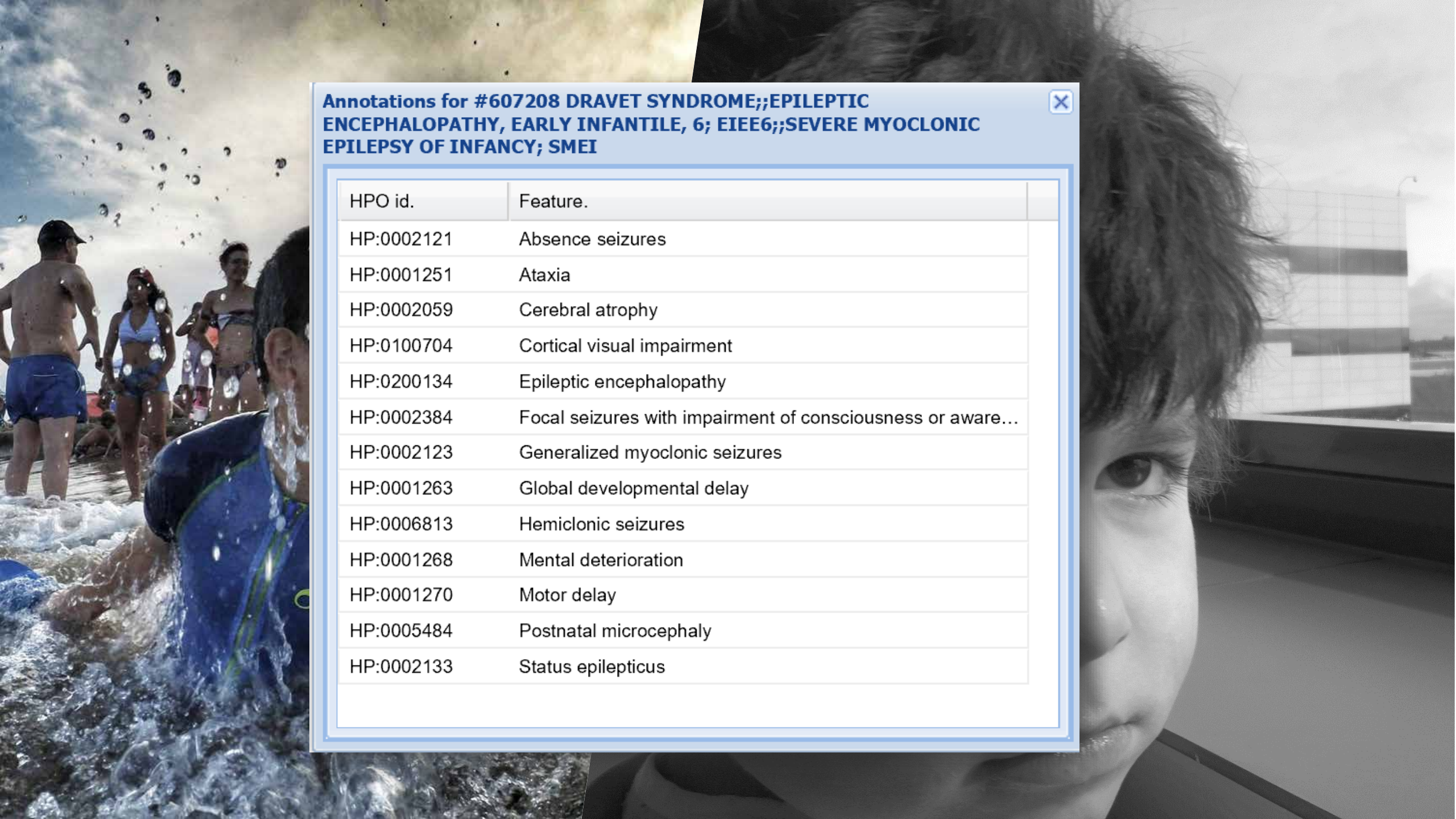
I attend this conference as an individual expert, and do not represent the
EMA. The views expressed here are my personal views, and may not be understood or quoted as being made on behalf of the EMA or reflecting the position of the COMP

CV

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/islaj_CV.pdf

DOI

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/islaj_DI.pdf



Annotations for #607208 DRAVET SYNDROME;;EPILEPTIC ENCEPHALOPATHY, EARLY INFANTILE, 6; EIEE6;;SEVERE MYOCLONIC EPILEPSY OF INFANCY; SMEI

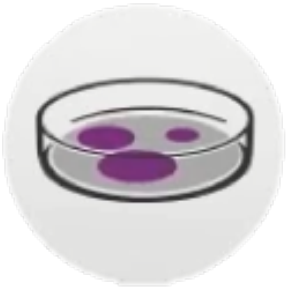
HPO id.	Feature.
HP:0002121	Absence seizures
HP:0001251	Ataxia
HP:0002059	Cerebral atrophy
HP:0100704	Cortical visual impairment
HP:0200134	Epileptic encephalopathy
HP:0002384	Focal seizures with impairment of consciousness or aware…
HP:0002123	Generalized myoclonic seizures
HP:0001263	Global developmental delay
HP:0006813	Hemiclonic seizures
HP:0001268	Mental deterioration
HP:0001270	Motor delay
HP:0005484	Postnatal microcephaly
HP:0002133	Status epilepticus

¿Pacientes?

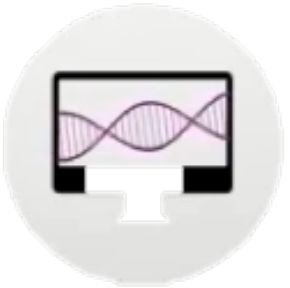
Somos **im**pacientes

Investigación

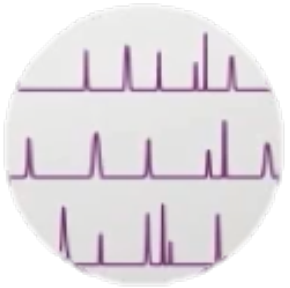
Tecnología



Modelos



Diagnóstico



Fenotipado
Genotipado

Registro

Medicamentos huérfanos

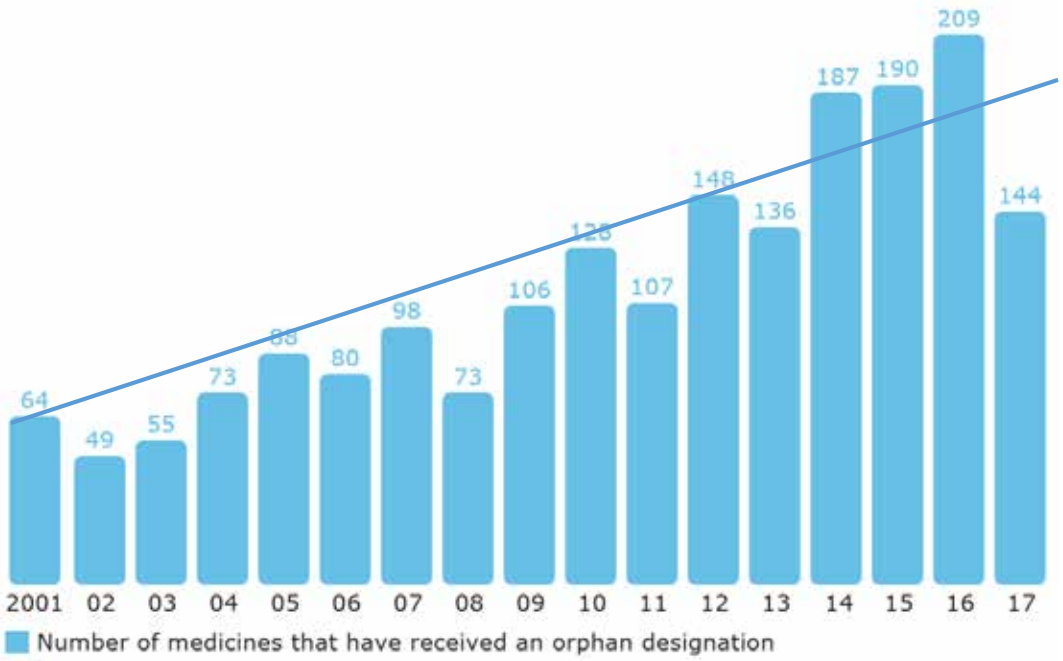
2008

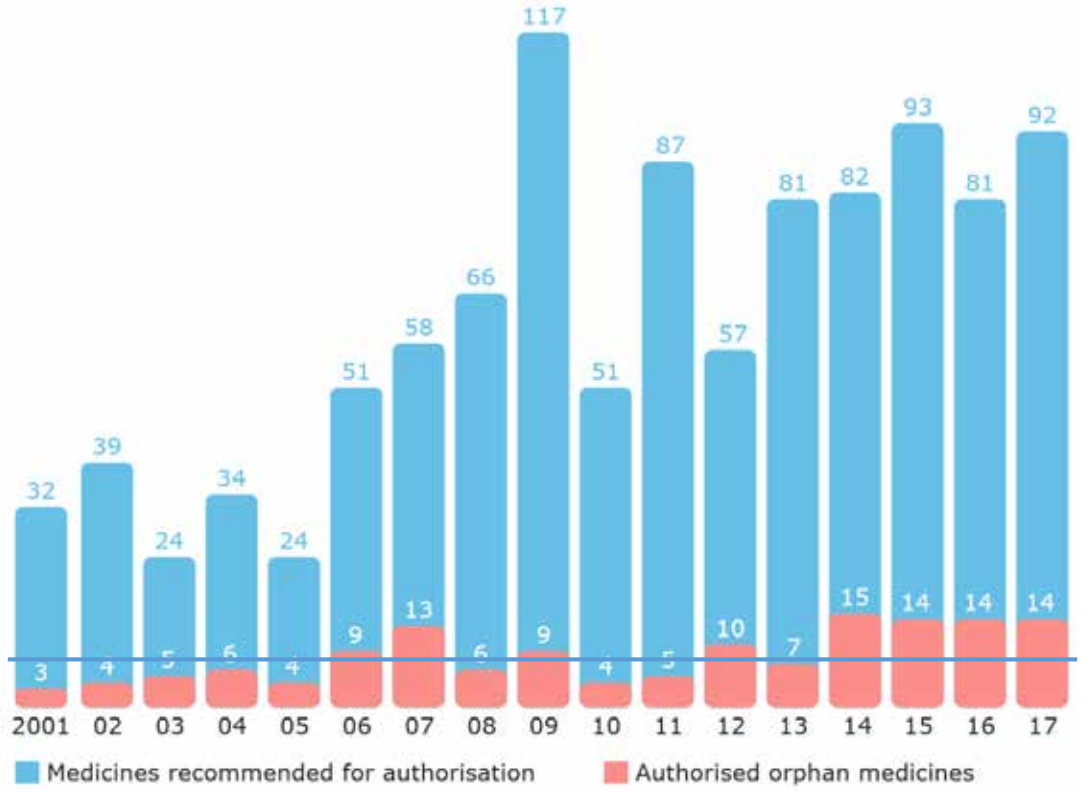
2018

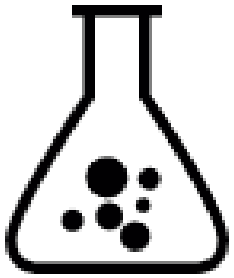
VPA+CLB+STP

VPA+CLB+STP

CBD / FEN





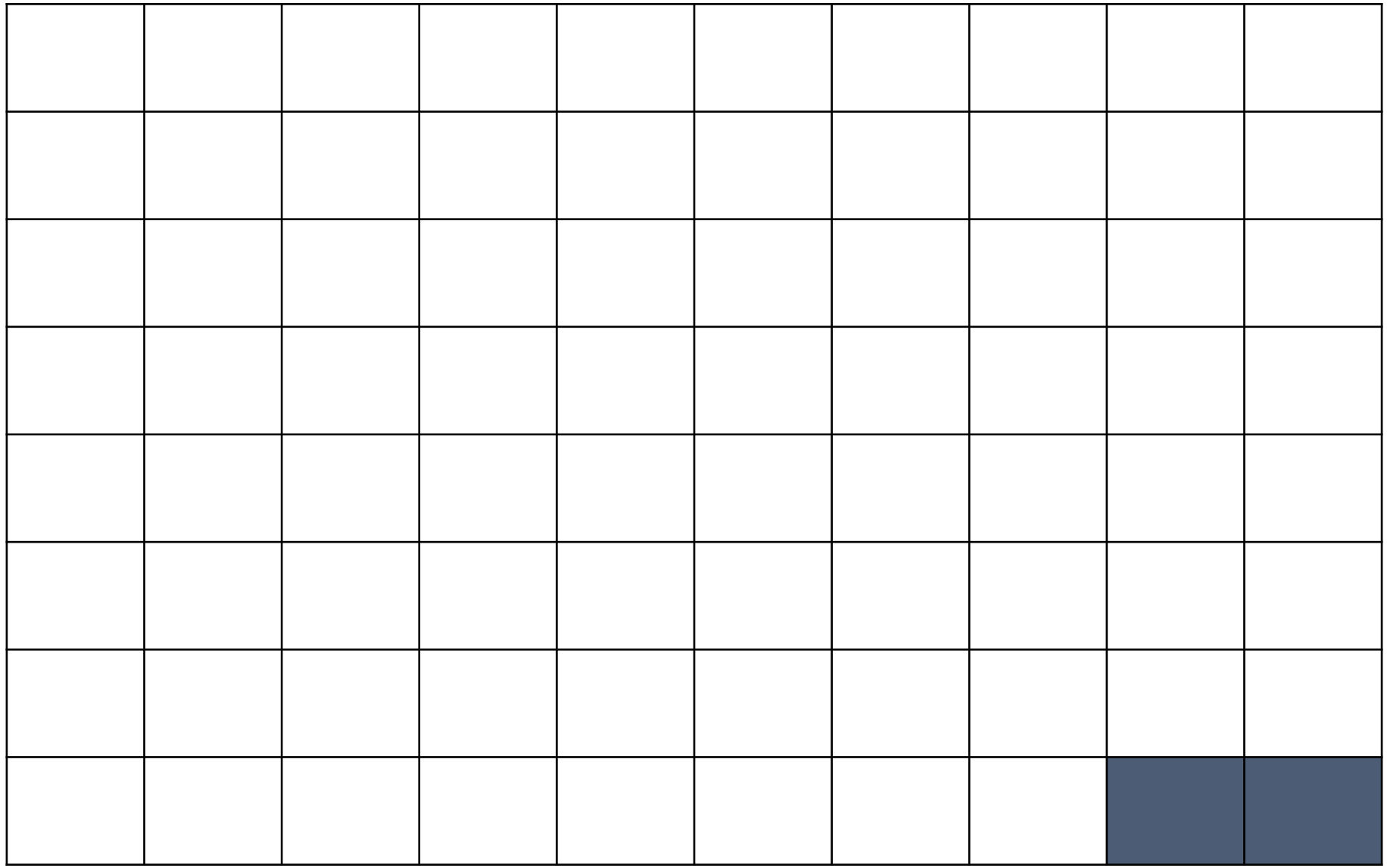


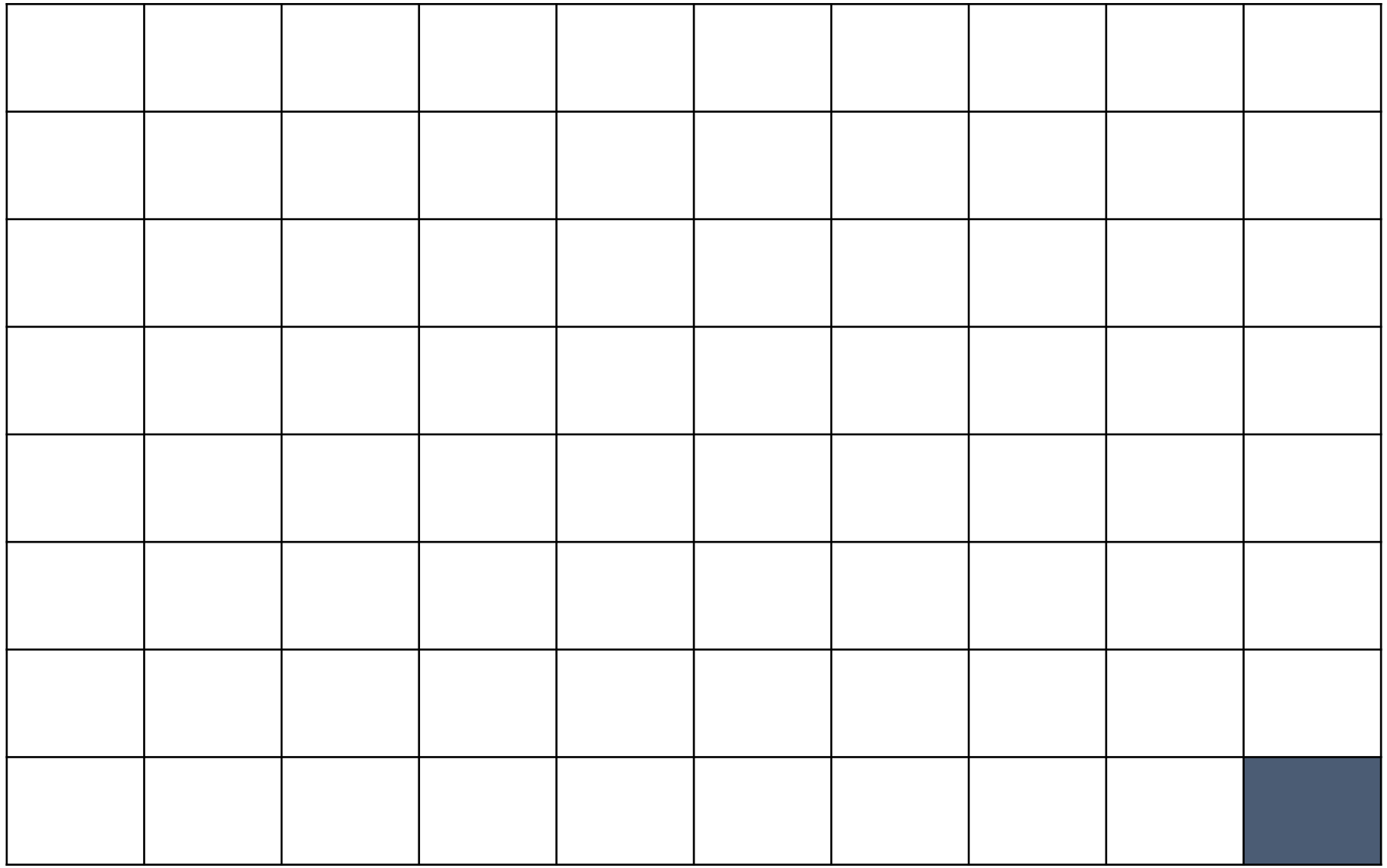
www.ema.europa.eu/docs/en_GB/document_library/Leaflet/2017/12/WC500240710.pdf

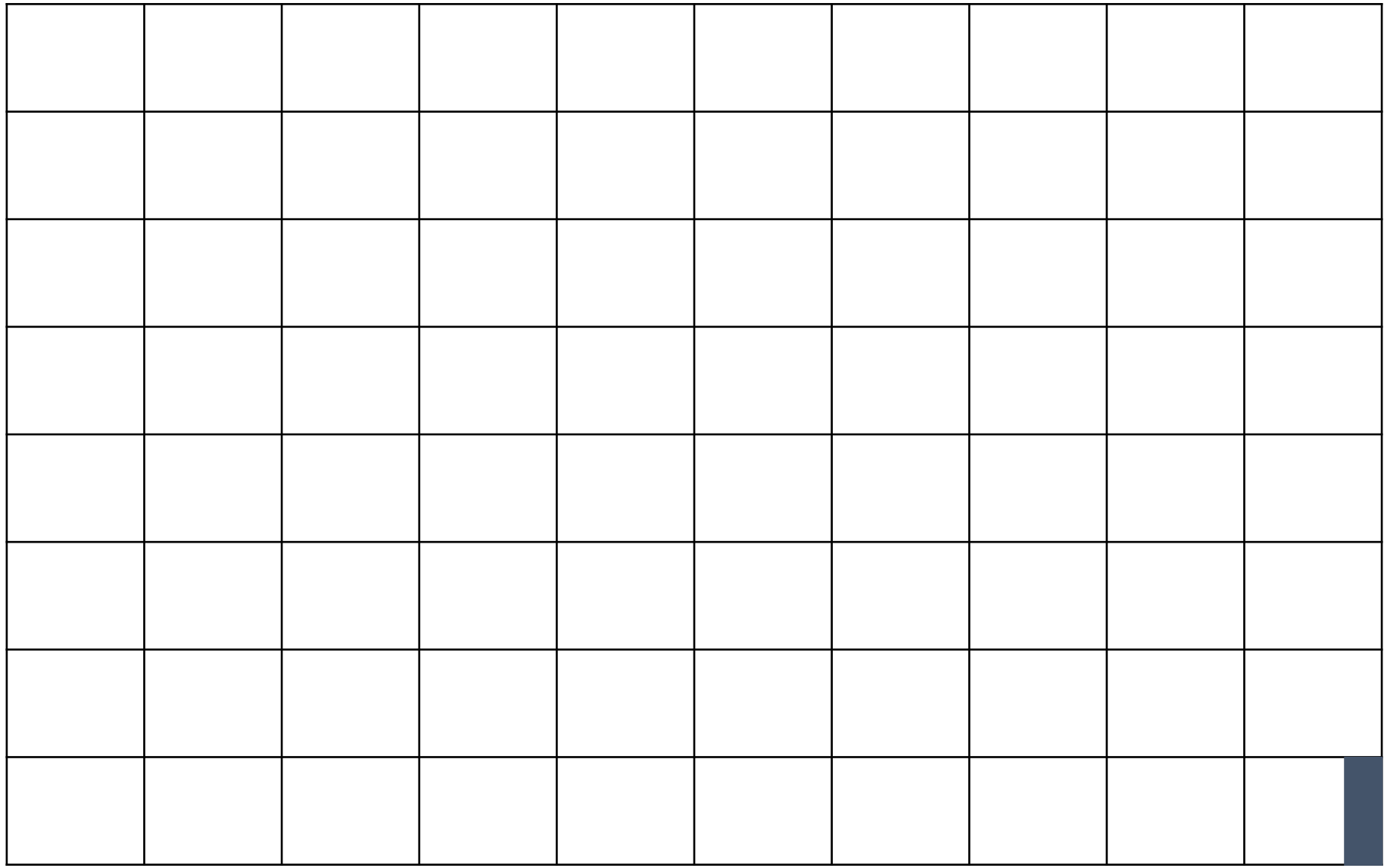
over 168 orphan medicines authorised in the EU

How orphan medicines reach patients

Once an orphan medicine is authorised by the European Commission, it can be marketed in all EU Member States. However, availability and reimbursment are subject to review by the relevant national authorities.







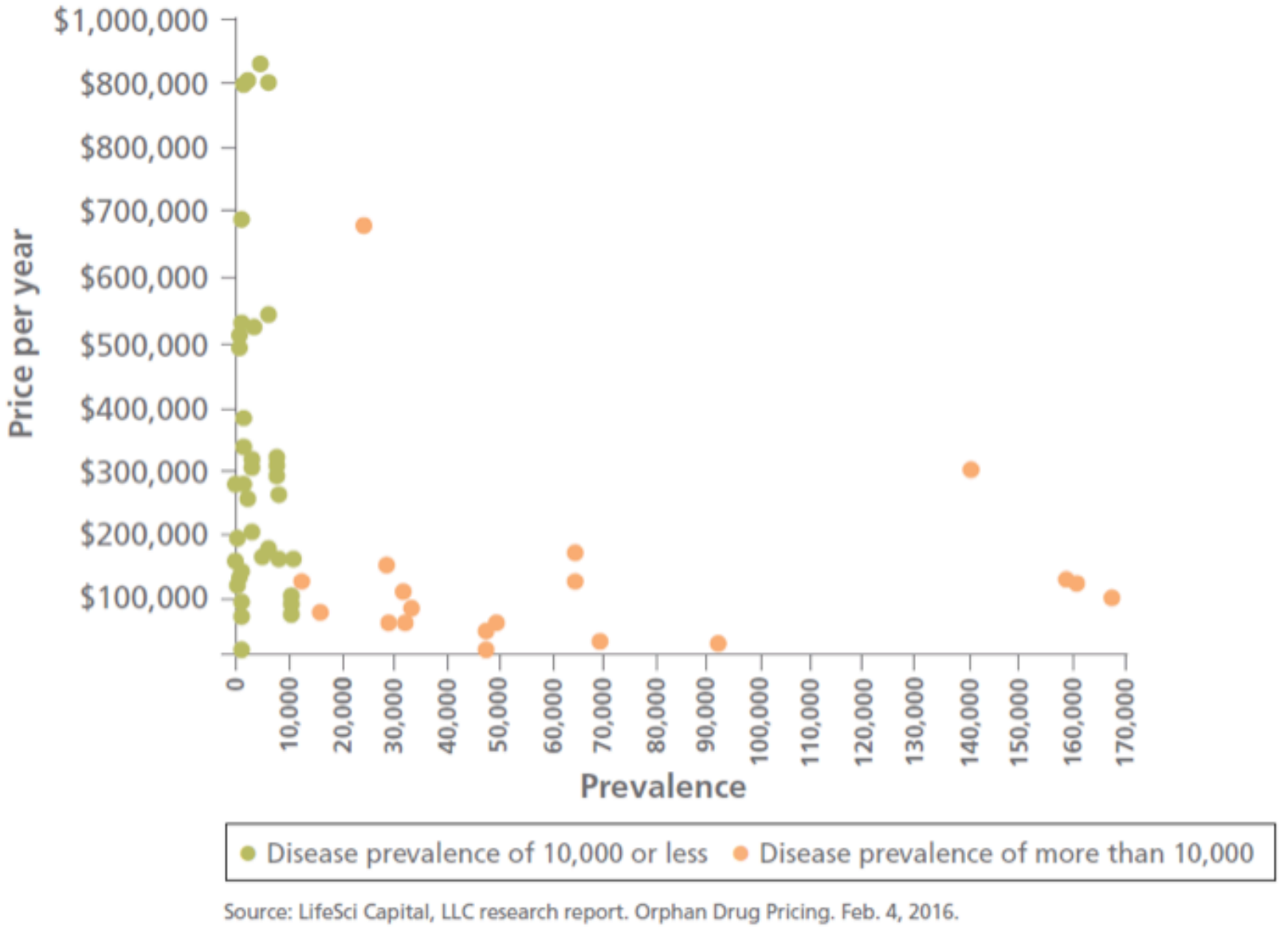
$

6.000.000.000.000

$

http://www.imf.org/en/Data

Orphan drug pricing versus disease prevalence



Source: LifeSci Capital, LLC research report. Orphan Drug Pricing. Feb. 4, 2016.

https://www.optum.com/resources/library/coming-to-grips-with-300000-drugs.html

Top-selling "orphan" drugs

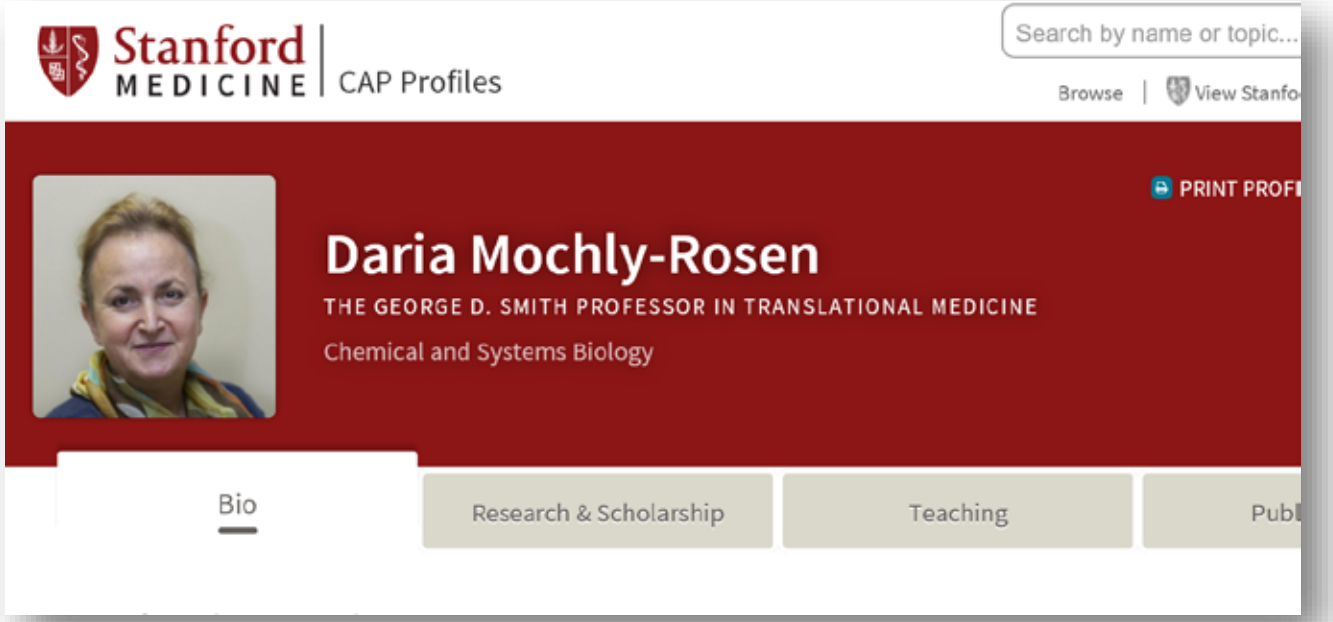
Rank	Brand	Orphan Utilization	Non-Orphan Utilization
1	Remicade	53%	47%
3	Rituxan	58%	42%
4	Avastin	31%	69%
7	Herceptin	3%	97%
8	Alimta	4%	96%
9	Gammagard	0%	100%
10	Gamunex	42%	58%
14	Xgeva	0%	100%
15	Velcade	92%	8%
17	Erbitux	36%	64%
18	Procrit	0%	100%
19	Soliris	76%	24%
20	Zometa	1%	99%
23	Orencia	0%	100%
24	Sandostatin	70%	30%
25	Abraxane	15%	85%

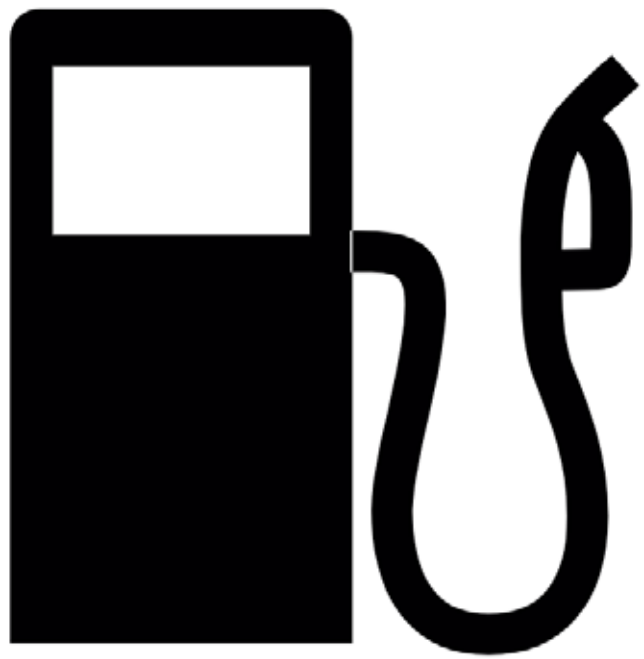
Source: MagellanRx Management, "Medical Pharmacy Trend Report 2013

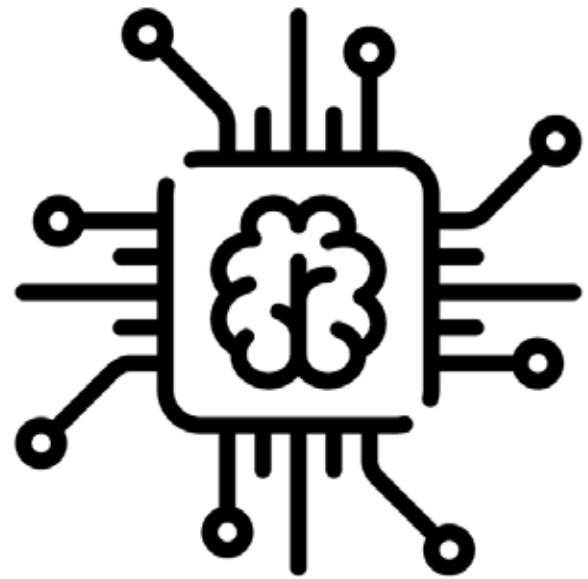
https://www.optum.com/resources/library/coming-to-grips-with-300000-drugs.html

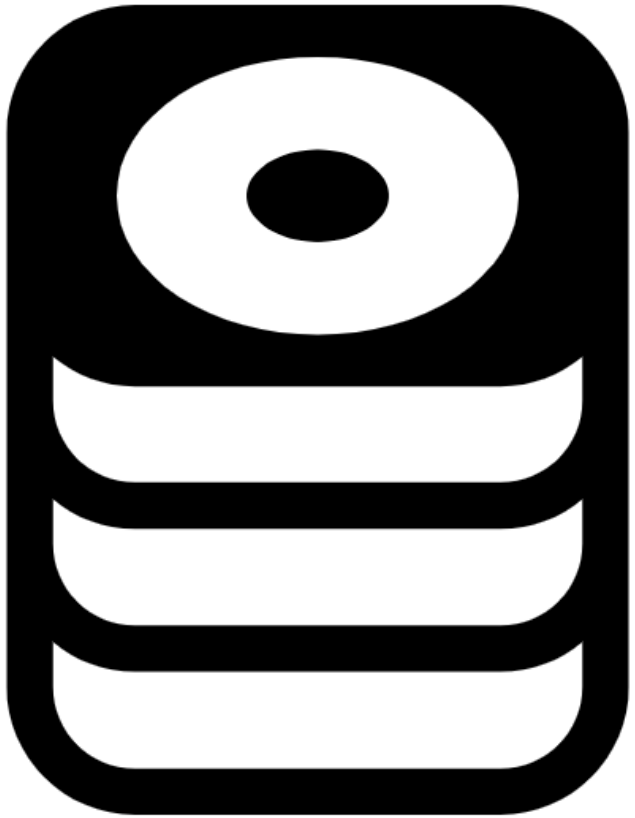
“When we're making drugs, we have to be better than nature”

“Drug development takes time and money because it's difficult and failure is a strong possibility”

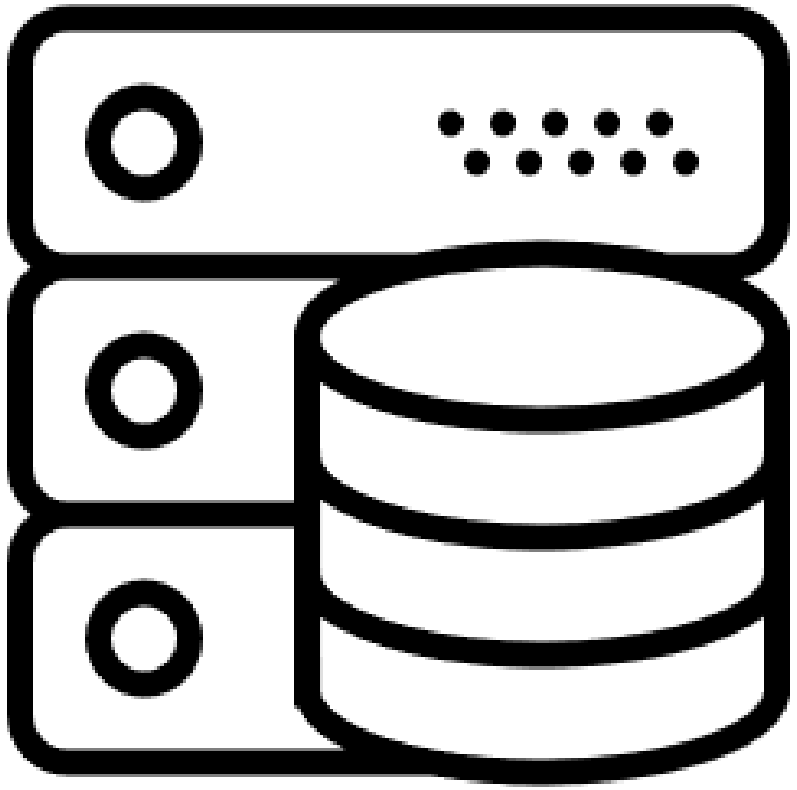








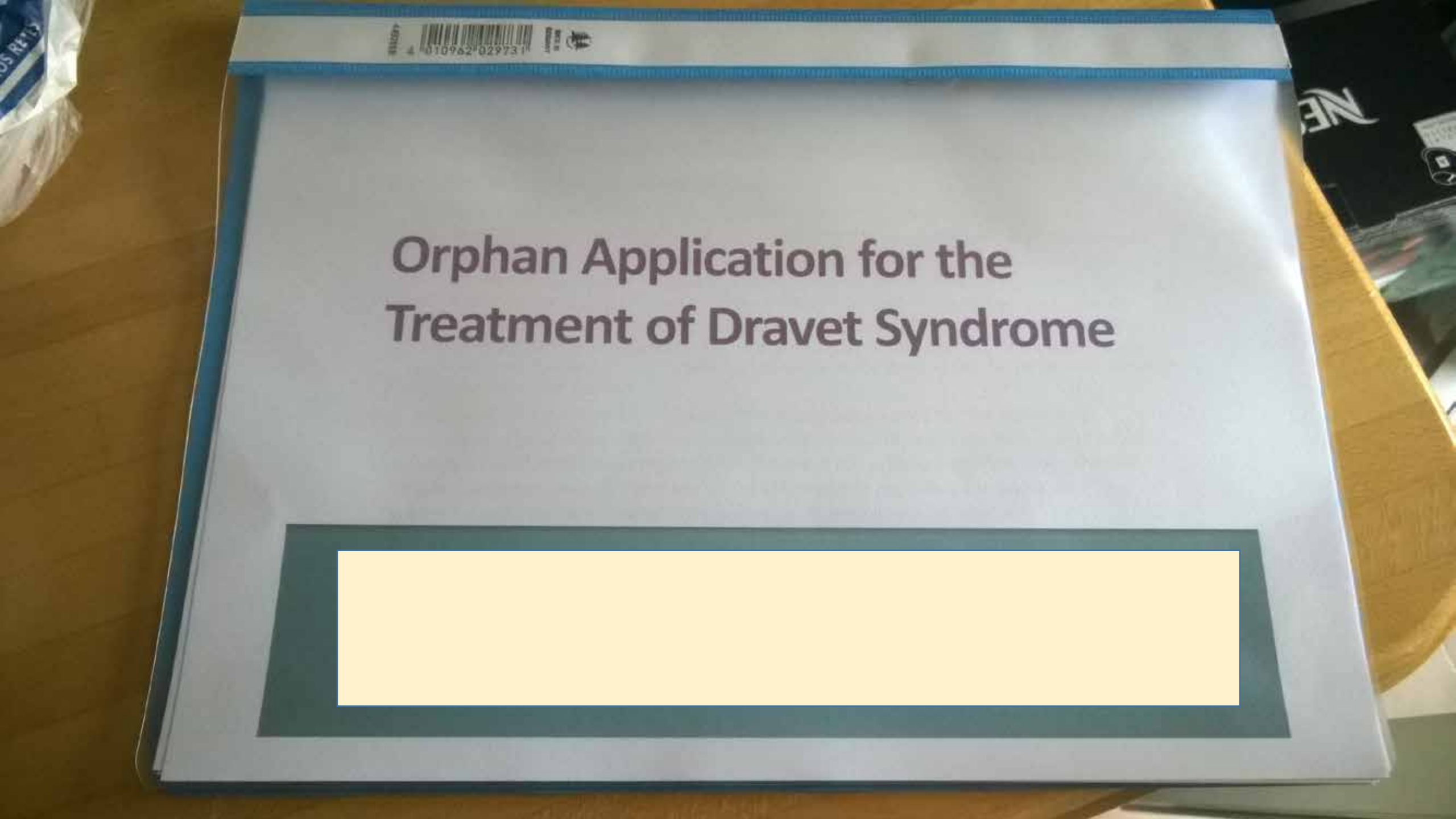
Los datos



El papel de los pacientes

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH


CANARY WHARF
ubilee line
astbound
latform 2
Canary Wharf
North Greenwich
Canning Town
West Ham
Stratford

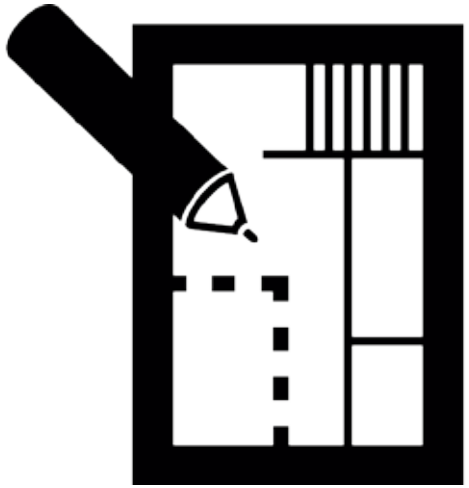

Orphan Application for the
Treatment of Dravet Syndrome


cucina

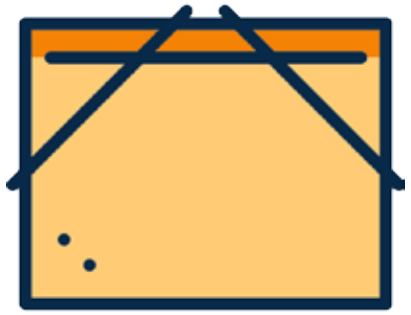
Scientific advice and protocol assistance

Consejo para **ensayo clínico**

Criterios de inclusion y exclusión




DIACOMIT® 500 mg
Gélules
Harde capsules
Hartkapseln
Enthält Natrium.
Vor Anwendung
Zum Einnehmen.
Kapseln unzerkaut mit
Arzneimittel für Kinder
aufbewahren.
In der Originalverpackung aufbewahren, um
den Inhalt vor Licht zu schützen.
Verschreibungspflichtig.



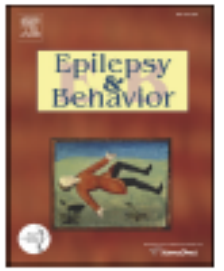
SurveyMonkey

Data from 274 patients in just one week

Contents lists available at ScienceDirect

Epilepsy & Behavior

journal homepage: www.elsevier.com/locate/yebeh



The European patient with Dravet syndrome: Results from a parent-reported survey on antiepileptic drug use in the European population with Dravet syndrome

Luis Miguel Aras, Julián Isla, Ana Mingorance-Le Meur *

Dravet Syndrome Foundation Spain, Madrid, Spain

ARTICLE INFO

Article history:
Received 16 October 2014
Revised 26 November 2014
Accepted 4 December 2014
Available online xxxx

Keywords:
Dravet syndrome
Childhood epilepsy
Antiepileptic drug
Orphan drug
Stiripentol
Clinical trials

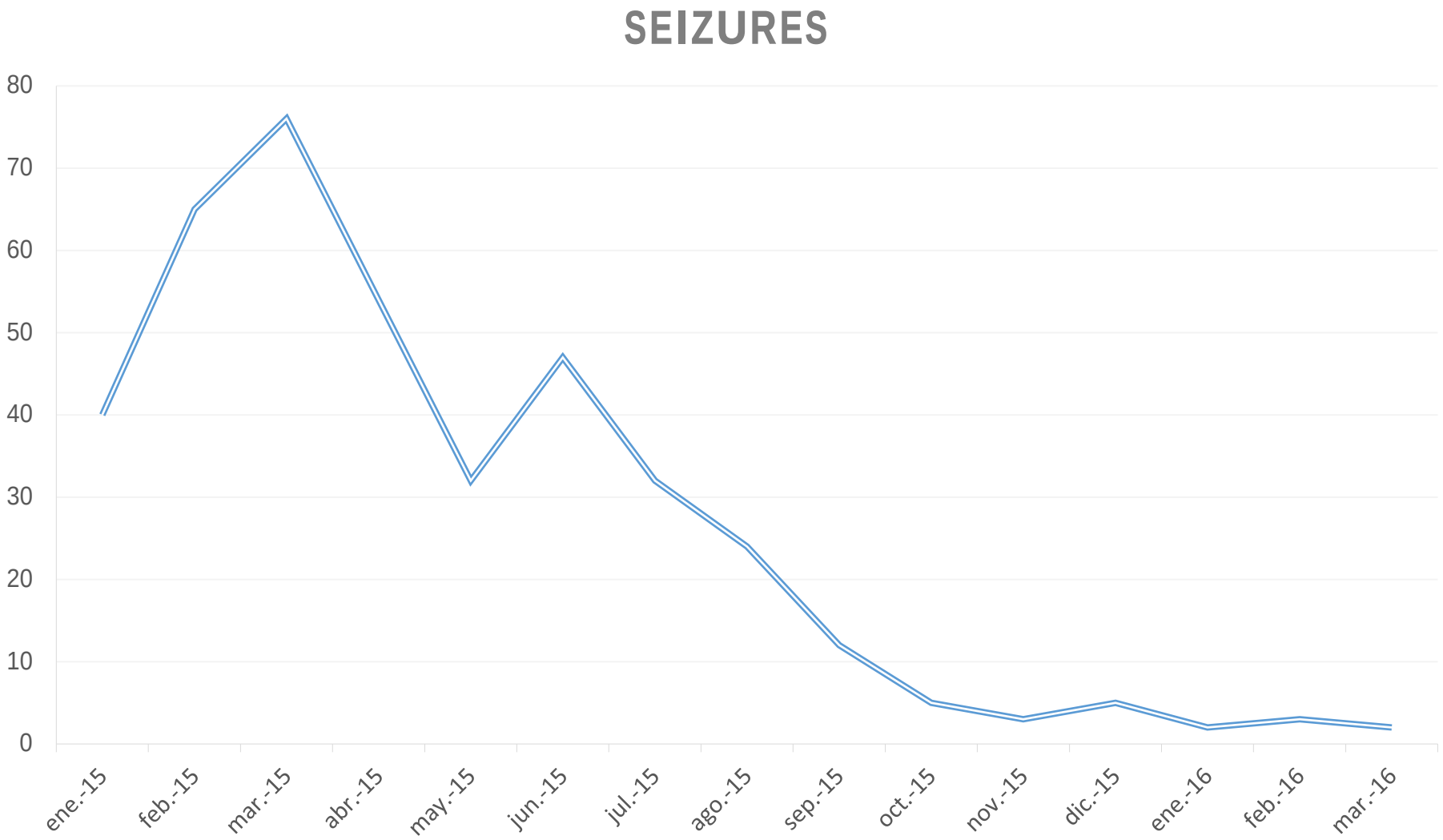
ABSTRACT

Dravet syndrome is a rare form of epilepsy largely refractory to current antiepileptic medications. The only precedents of randomized placebo-controlled trials in Dravet syndrome are the two small trials that led to the approval of stiripentol. With the arrival of new clinical trials for Dravet syndrome, we sought to determine the characteristics of the patient population with Dravet syndrome in Europe today, which has possibly evolved subsequent to the approval of stiripentol and the ability to diagnose milder clinical cases via genetic testing. From May to June 2014, we conducted an online parent-reported survey to collect information about the demographics, disease-specific clinical characteristics, as well as current and past use of antiepileptic medications by European patients with Dravet syndrome. We present data from 274 patients with Dravet syndrome from 15 European countries. Most patients were between 4 and 8 years of age, and 90% had known mutations in *SCN1A*. Their epilepsy was characterized by multiple seizure types, although only 45% had more than 4 tonic–clonic seizures per month on average. The most common drug combination was valproate, clobazam, and stiripentol, with 42% of the total population currently taking stiripentol. Over a third of patients with Dravet syn-

Las consecuencias en el diseño

¿PUEDO PARTICIPAR?

Estudios 1501 y 1502	Estudio 1504
Los participantes recibirán de manera aleatoria (al azar): ZX008 durante un máximo de 16 semanas O Un placebo (un medicamento "simulado") durante un máximo de 16 semanas Los participantes tendrán el doble de	Los participantes recibirán de manera aleatoria (al azar): ZX008 más otro medicamento del estudio durante un máximo de 19 semanas O Un placebo más otro medicamento del estudio durante un máximo de 19 semanas

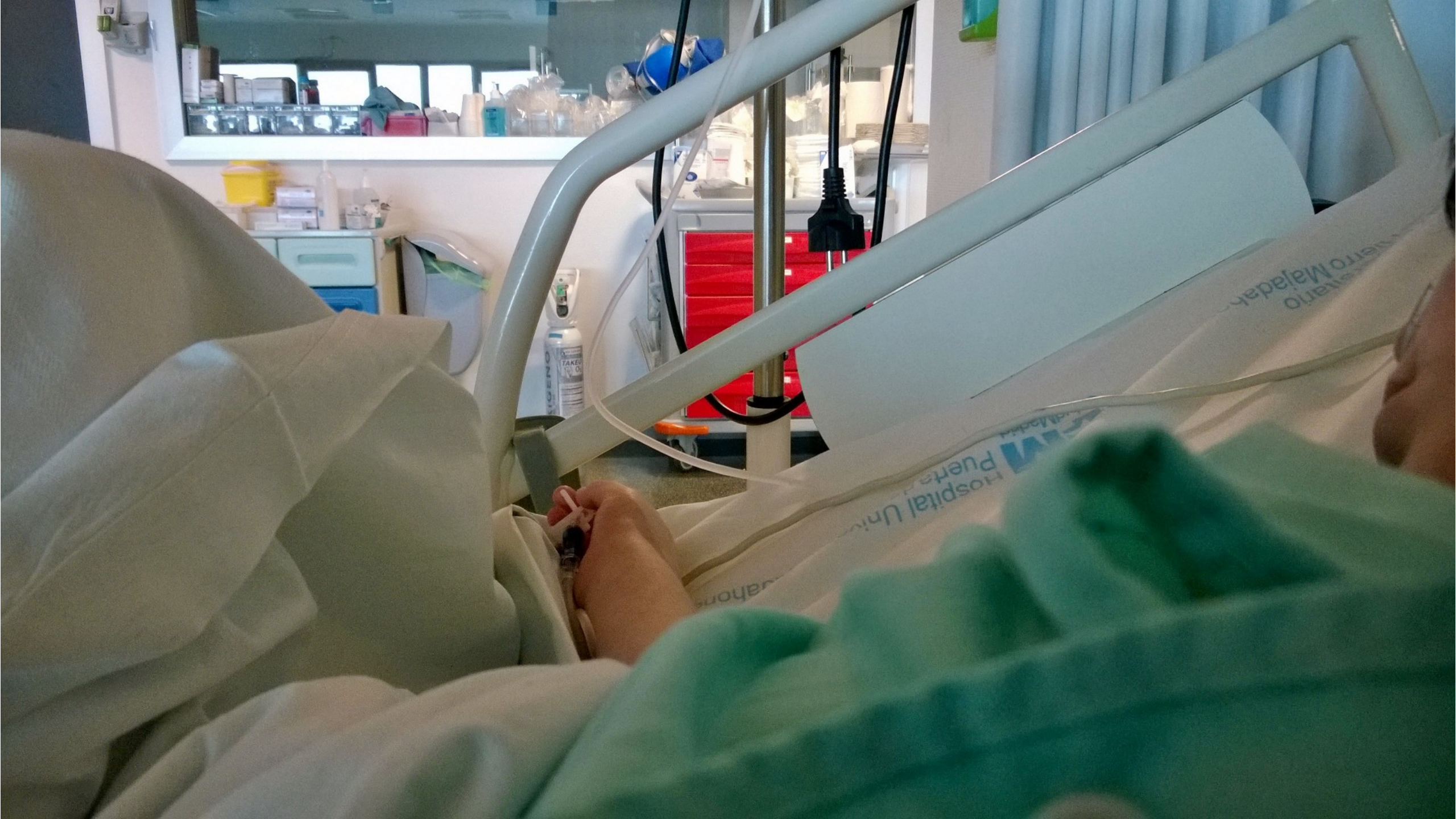

SEIZURES
80
70
60
50
40
30
20
10
0
ene.-15
feb.-15
mar.-15
abr.-15
may.-15
jun.-15
jul.-15
ago.-15
sep.-15
oct.-15
nov.-15
dic.-15
ene.-16
feb.-16
mar.-16

Crear sistemas que nos ayuden a tomar decisiones

La cabina de un Lockheed Constellation

La cabina de un Airbus 350


dreamstime



Lo que sabemos es una gota de agua; lo que ignoramos es el océano.

Los hombres construimos demasiados muros y no suficientes puentes.

Si he hecho descubrimientos invaluables ha sido más por tener paciencia que cualquier otro talento.

Si consigo ver más lejos es porque he conseguido auparme a hombros de gigantes.

Isaac Newton

Gracias

julian.isla@foundation29.org
@julianig

foundation twenty-nine