



Integración del paciente en la investigación y ensayos clínicos

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Federación Europea de Síndrome de Dravet
Fundación 29



Microsoft Consulting Services. AI



Presidente y fundador



draveteurope
Dravet Syndrome European Federation

Director Científico



TAG. Therapeutic Advisory Group

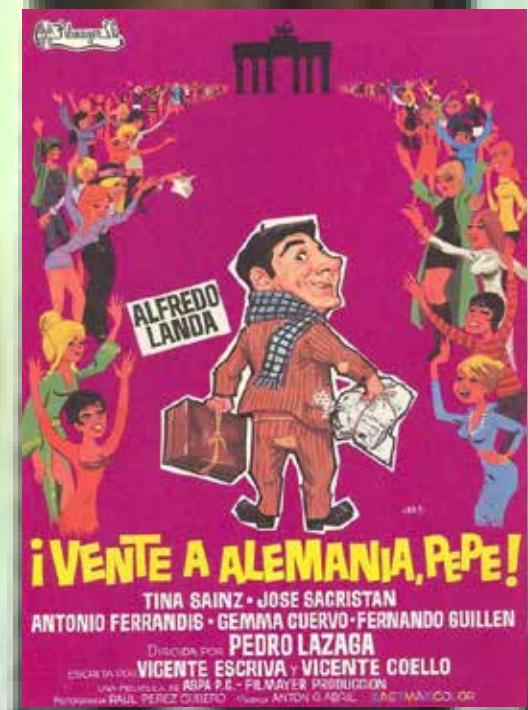


Miembro del COMP



Comité científico asesor







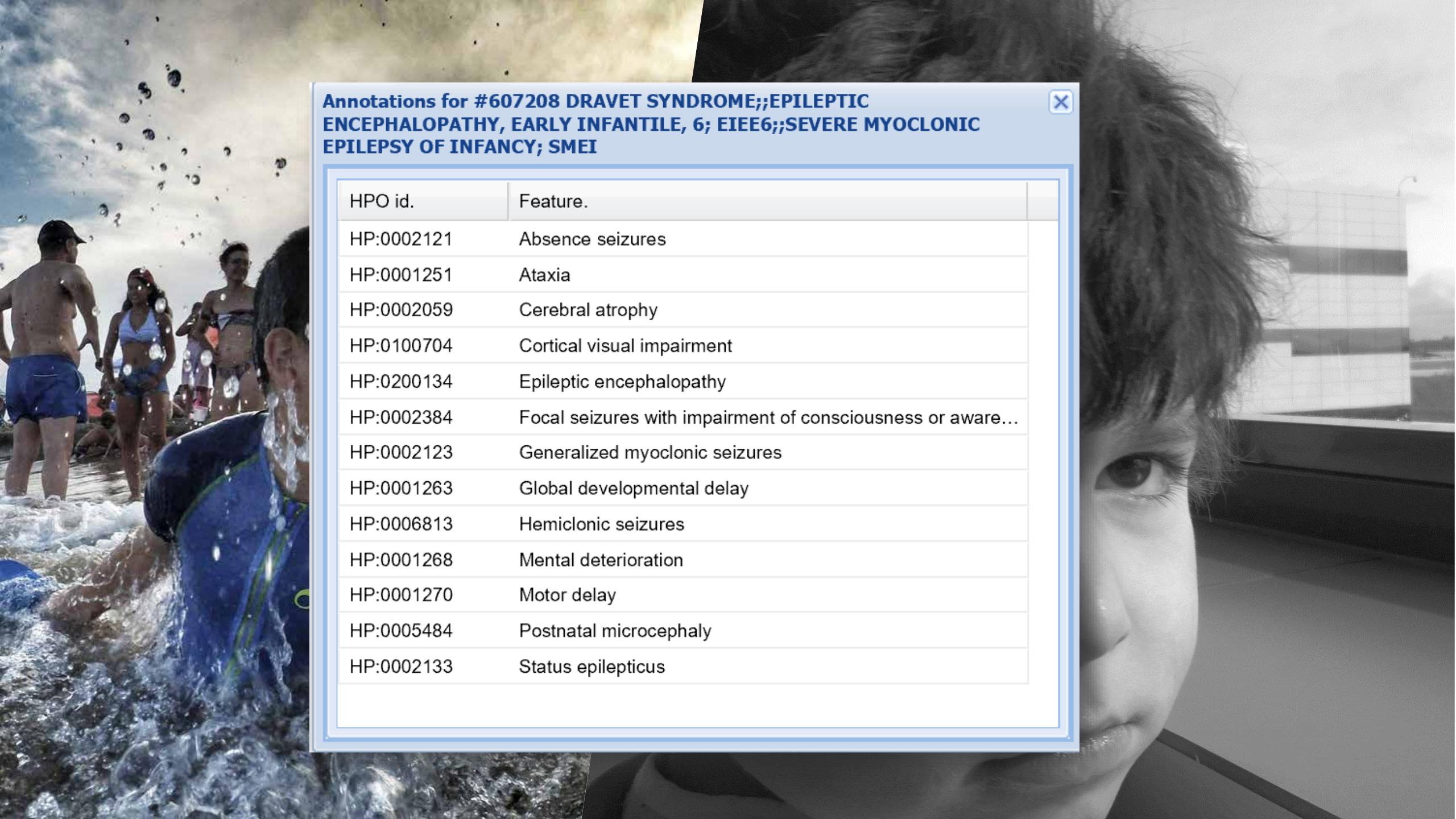
I attend this conference as an individual expert, and do not represent the EMA. The views expressed here are my personal views, and may not be understood or quoted as being made on behalf of the EMA or reflecting the position of the COMP

CV

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/7islaj_CV.pdf

DOI

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/7islaj_DI.pdf



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

X

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?



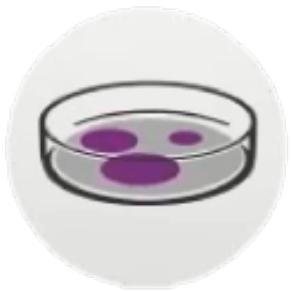
Somos impacientes



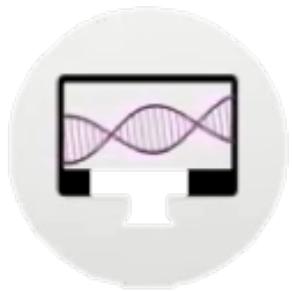
Investigación



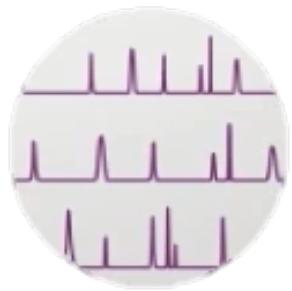
Tecnología



Modelos



Diagnóstico



Fenotipado
Genotipado



Registro



Medicamentos huérfanos

2008

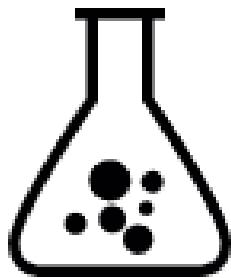
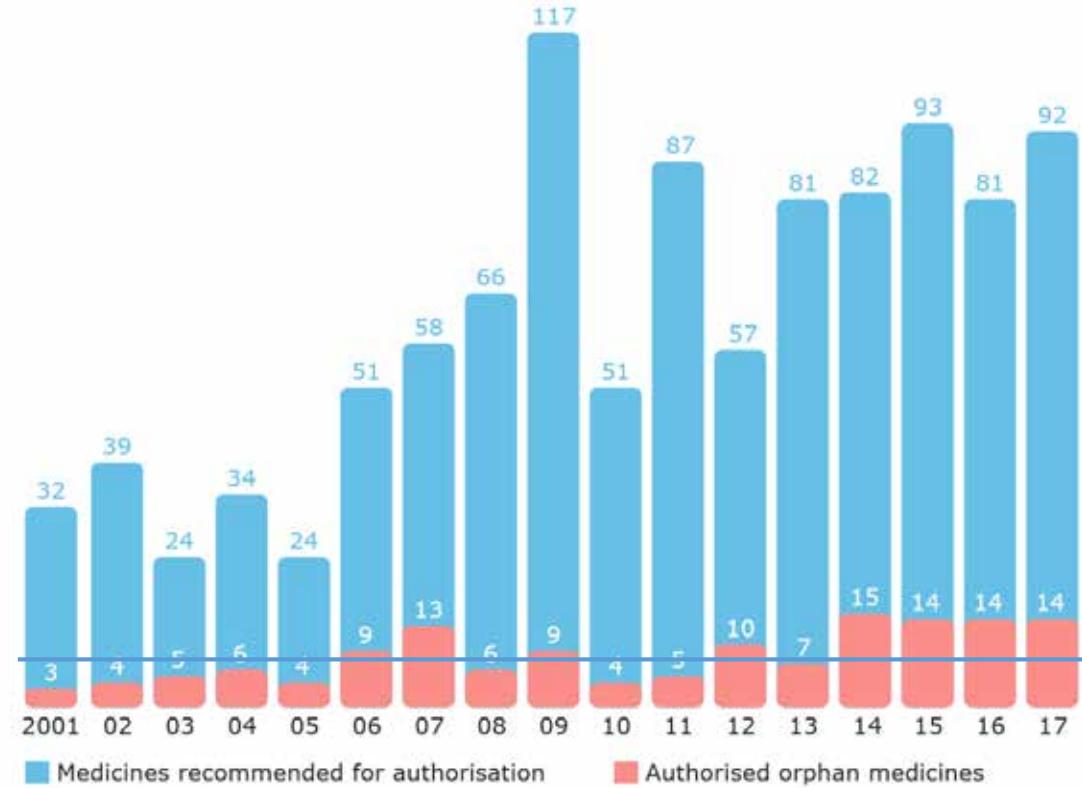
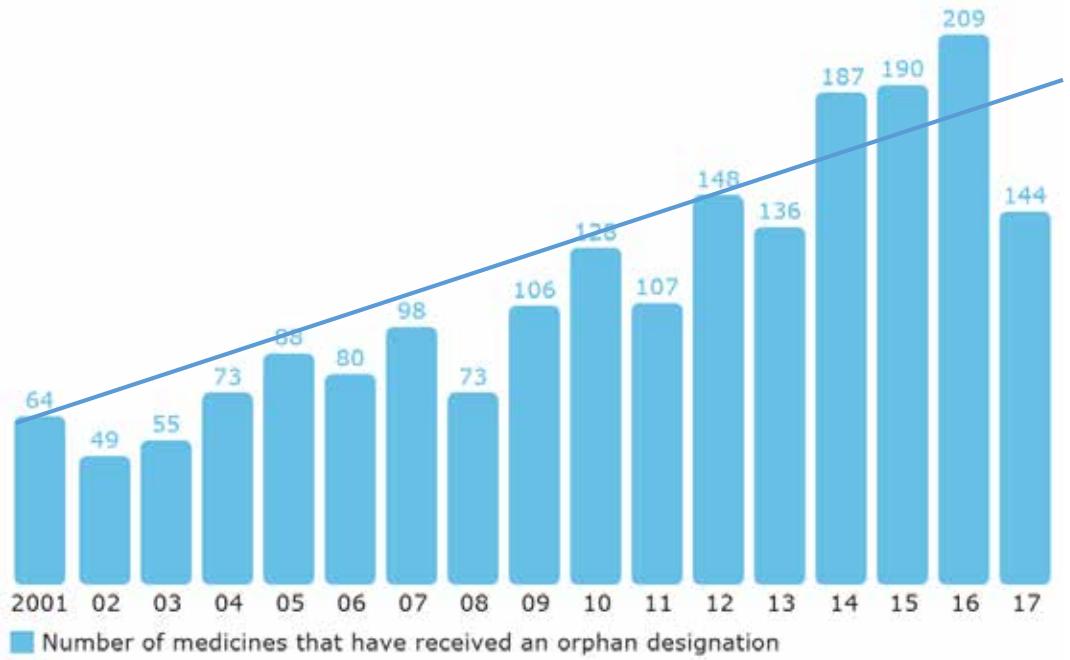
VPA+CLB+STP

2018

VPA+CLB+STP

CBD / FEN





over **168** orphan medicines
authorised in the EU

How orphan medicines reach patients

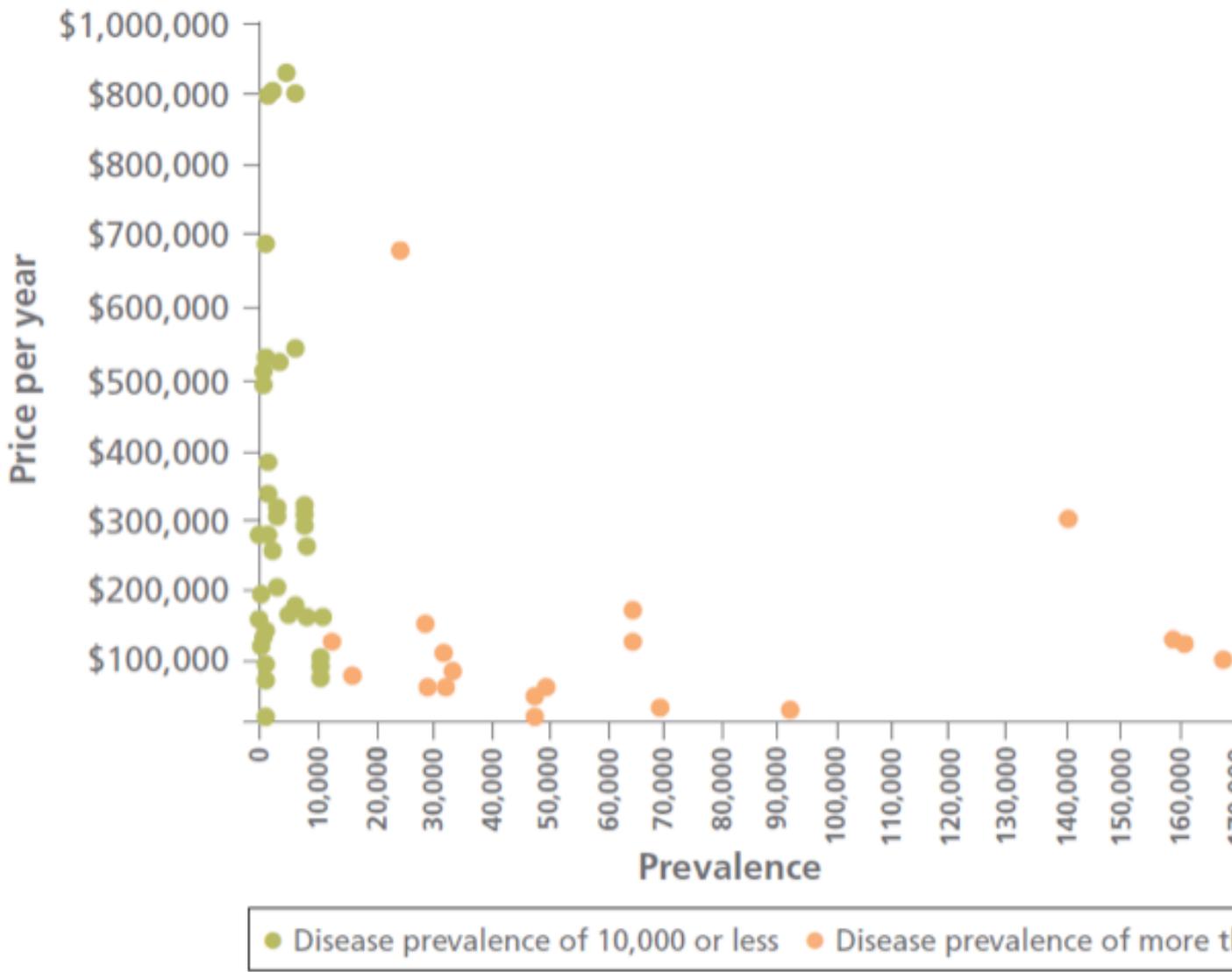
Once an orphan medicine is authorised by the European Commission, it can be marketed in all EU Member States. However, availability and reimbursement are subject to review by the relevant national authorities.

\$
6.000.000.000.000

\$



Orphan drug pricing versus disease prevalence



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

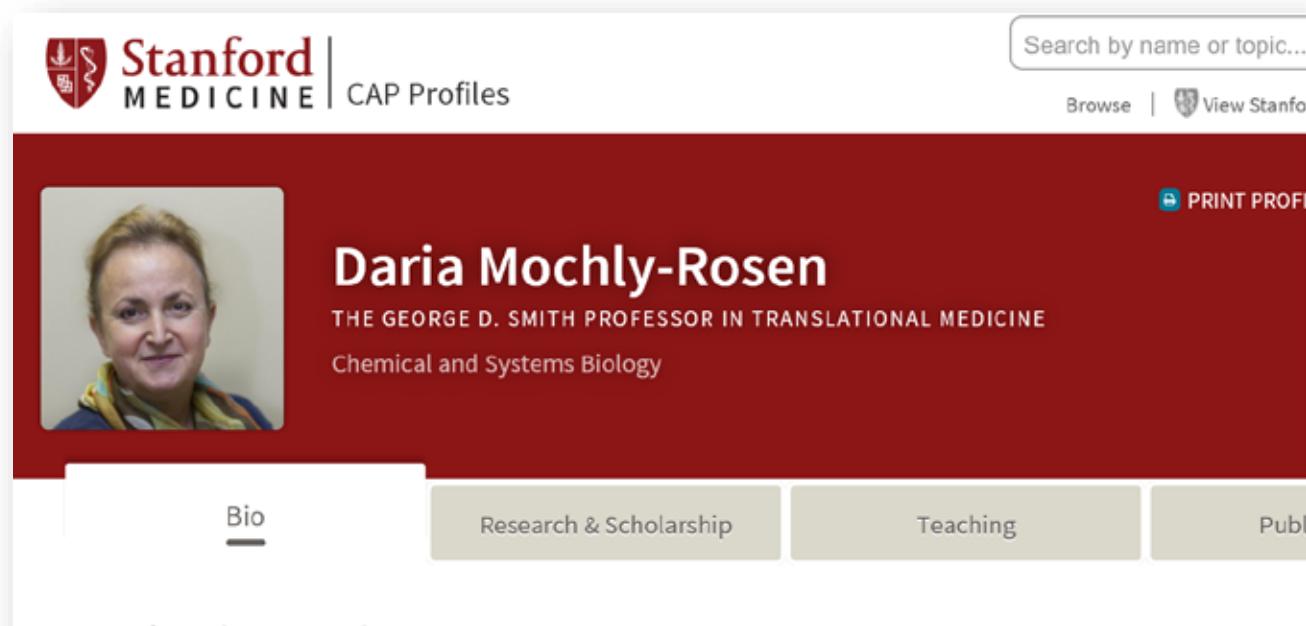
Top-selling “orphan” drugs

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%

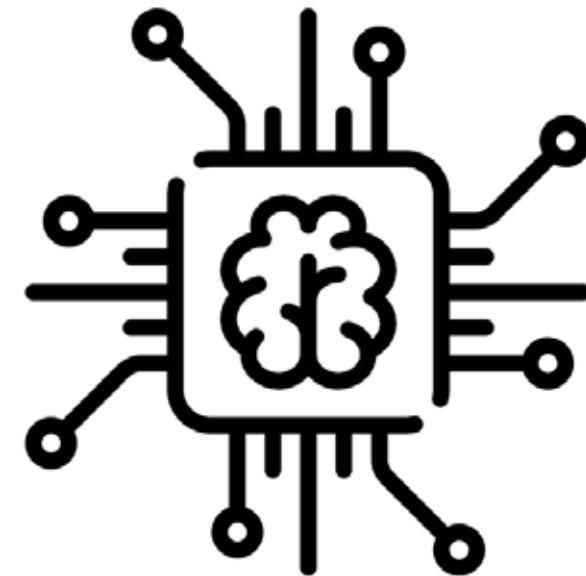
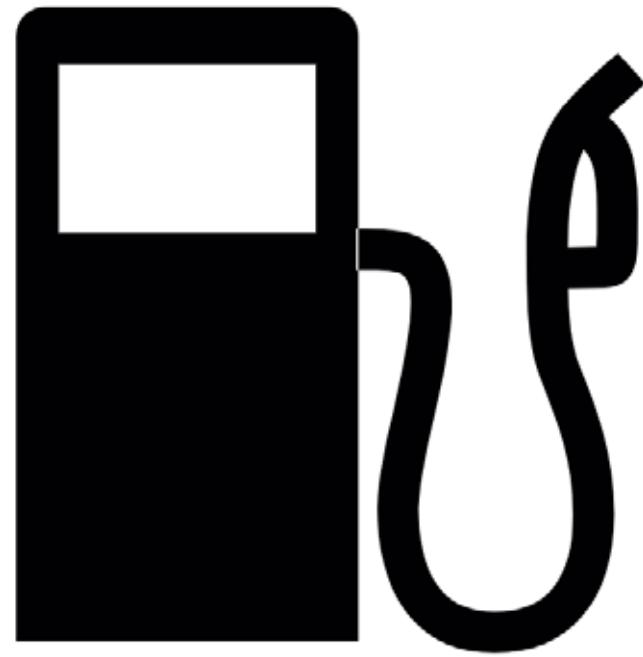
Source: MagellanRx Management, "Medical Pharmacy Trend Report 2013"

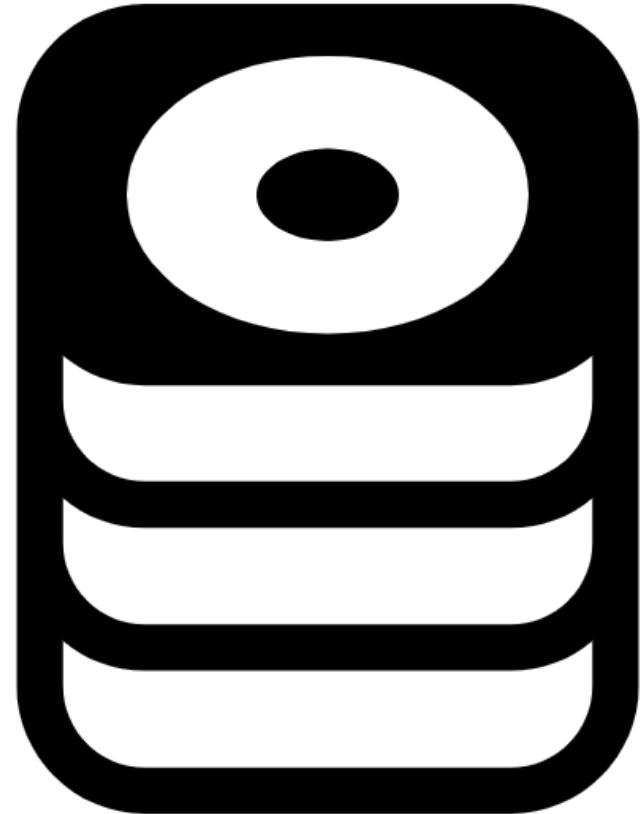
“When we're making drugs, we have to be better than nature”

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

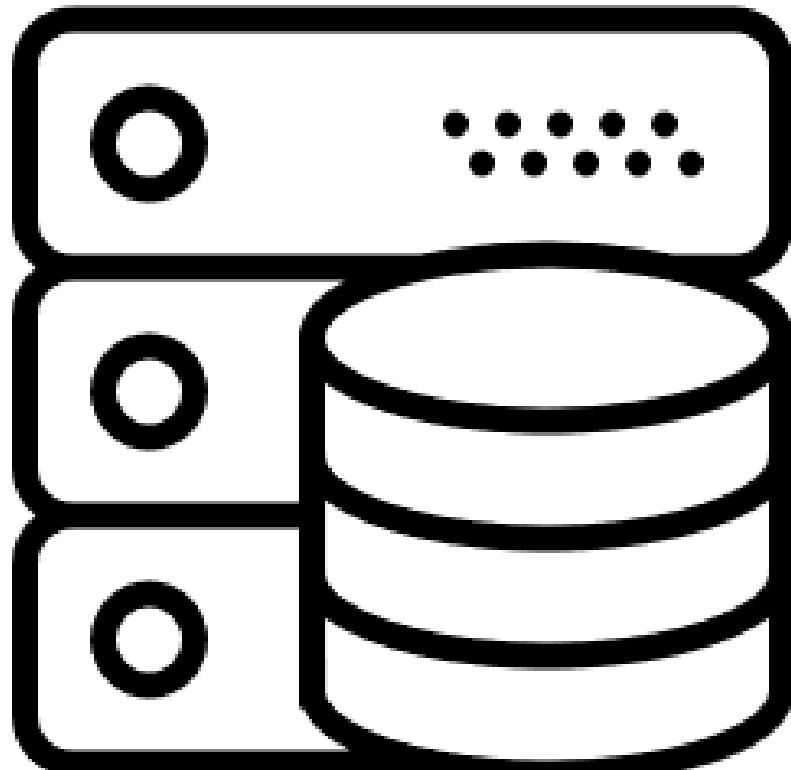


The image shows a screenshot of the Stanford Medicine CAP Profiles website. At the top left is the Stanford Medicine logo. To its right is the text "CAP Profiles". On the far right is a search bar with the placeholder "Search by name or topic...". Below the header, there is a red banner featuring a portrait of Daria Mochly-Rosen, a woman with blonde hair wearing a blue jacket over a yellow patterned scarf. To her right, her name "Daria Mochly-Rosen" is written in large white letters, followed by "THE GEORGE D. SMITH PROFESSOR IN TRANSLATIONAL MEDICINE" and "Chemical and Systems Biology". Below the banner, there are four tabs: "Bio" (which is highlighted in white), "Research & Scholarship", "Teaching", and "Publications".





Los datos



El papel de los
pacientes



draveteurope

Dravet Syndrome European Federation



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Jubilee line
Eastbound
Platform 2

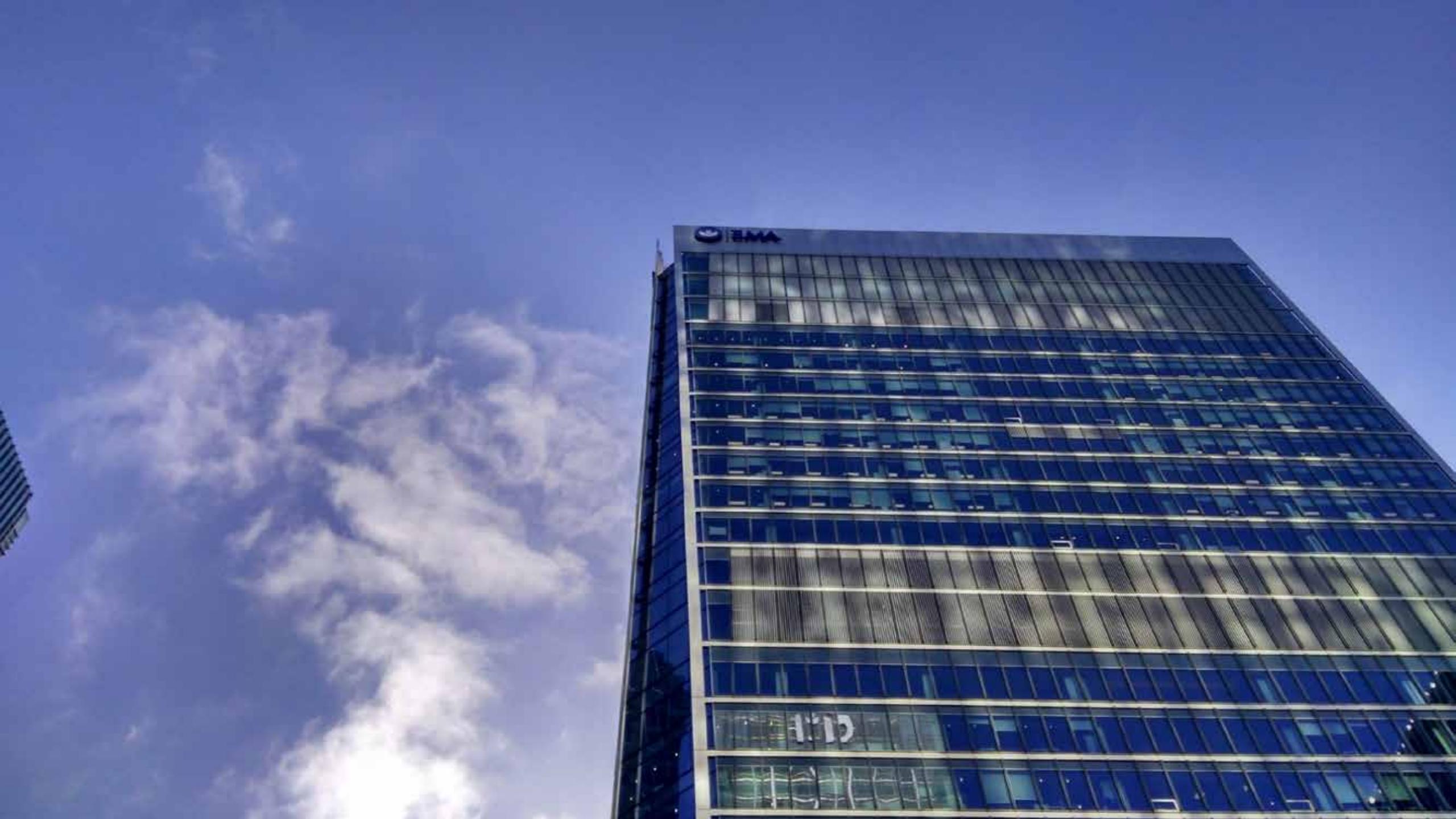
Canary Wharf
North Greenwich
Canning Town
West Ham
Stratford



CANARY WHARF

to Westbound Platform 1

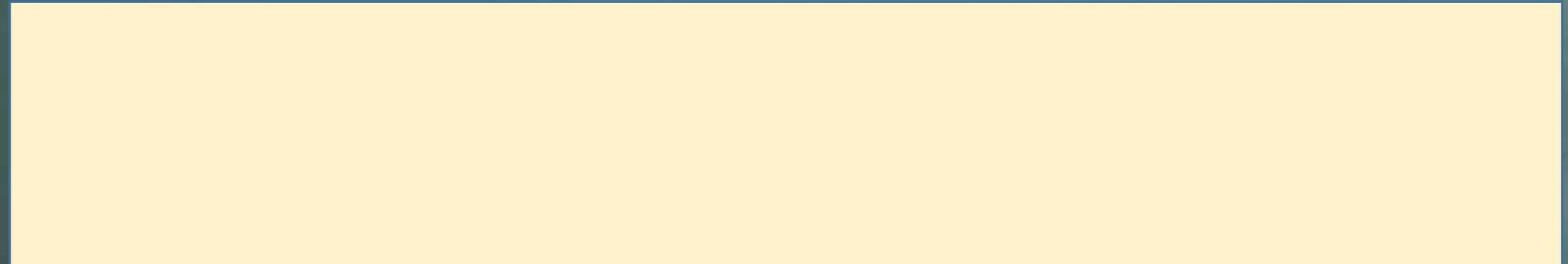






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Orphan Application for the Treatment of Dravet Syndrome





Scientific advice and protocol assistance

The screenshot shows the European Medicines Agency (EMA) website. At the top, there is a logo of a white bowl with a blue base, followed by the text "EUROPEAN MEDICINES AGENCY" and "SCIENCE MEDICINES HEALTH". To the right are links for "Text size: A A A", "Site-wide search" with a magnifying glass icon, and a "GO" button. There is also a link to "Search document library" with a magnifying glass icon. Below this is a "Follow us" section with icons for Twitter, RSS, and YouTube.

The main navigation menu includes "Home", "Find medicine", "Human regulatory" (which is highlighted in blue), "Veterinary regulatory", "Committees", "News & events", "Partners & networks", and "About us".

The left sidebar has a vertical list of committees: CHMP, PRAC, CVMP, COMP, HMPC, CAT, PDCO, "Working parties and other groups" (with a plus sign), and "CHMP" (with a minus sign). The "Working parties and other groups" item is currently expanded, showing "CHMP".

The main content area shows the breadcrumb navigation: "Home > Committees > Working parties and other groups > CHMP > Scientific Advice Working Party". The title "Scientific Advice Working Party" is displayed, along with "Email", "Print", "Help", and "Share" buttons. The text describes the Scientific Advice Working Party (SAWP) as a standing working party with the sole remit of providing scientific advice and protocol assistance, established by the Committee for Medicinal Products for Human Use (CHMP).

Below this, it states that the SAWP is a multidisciplinary group comprising a chairperson, 28 members including three from the Committee for Orphan Medicinal Products (COMP), one from the Paediatric Committee (PDCO), and one from the Committee for Advanced Therapies (CAT).

It also mentions that in the nomination process, a fair representation of areas of expertise is ensured, listing "non-clinical safety" and "pharmacokinetics".

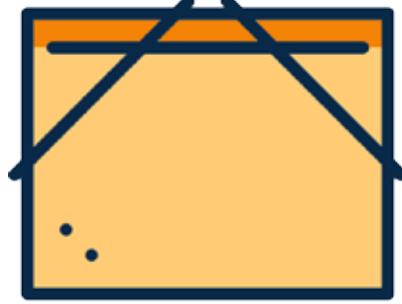
On the right side, there is a "Related information" section with a link to "Scientific advice and protocol assistance".

Consejo para ensayo clínico

Criterios de inclusión y exclusión



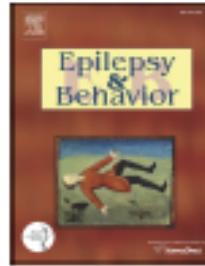




Data from 274 patients in just one week



Contents lists available at ScienceDirect



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



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Dravet Syndrome Foundation Spain, Madrid, Spain

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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

INFORMACIÓN SOBRE EL SÍNDROME DE DRAVET

INFORMACIÓN SOBRE LA INVESTIGACIÓN CLÍNICA

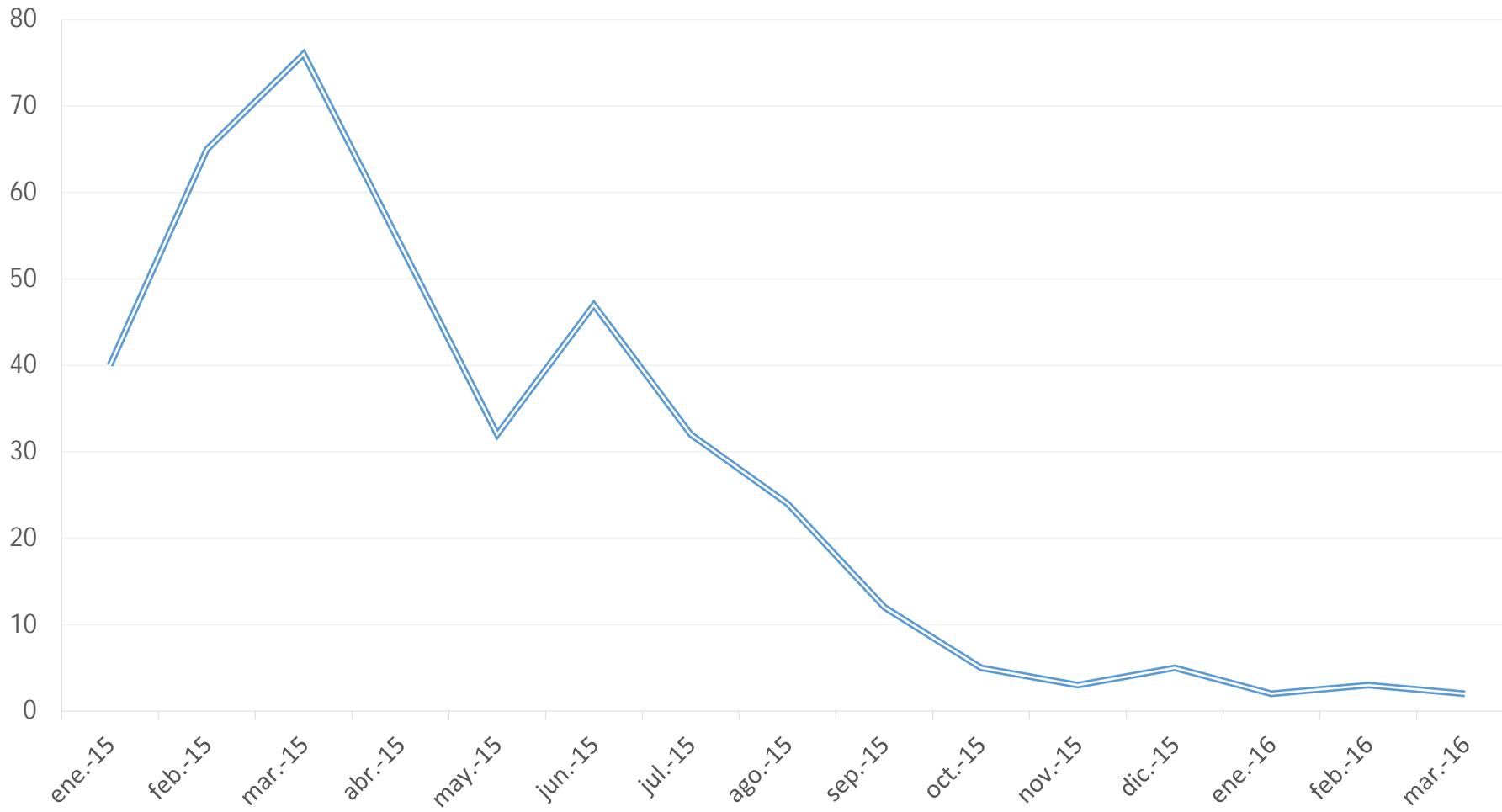
INFORMACIÓN SOBRE FAIRE

Medicamentos dirigidos contra el síndrome de Dravet.

¿PUEDO PARTICIPAR?

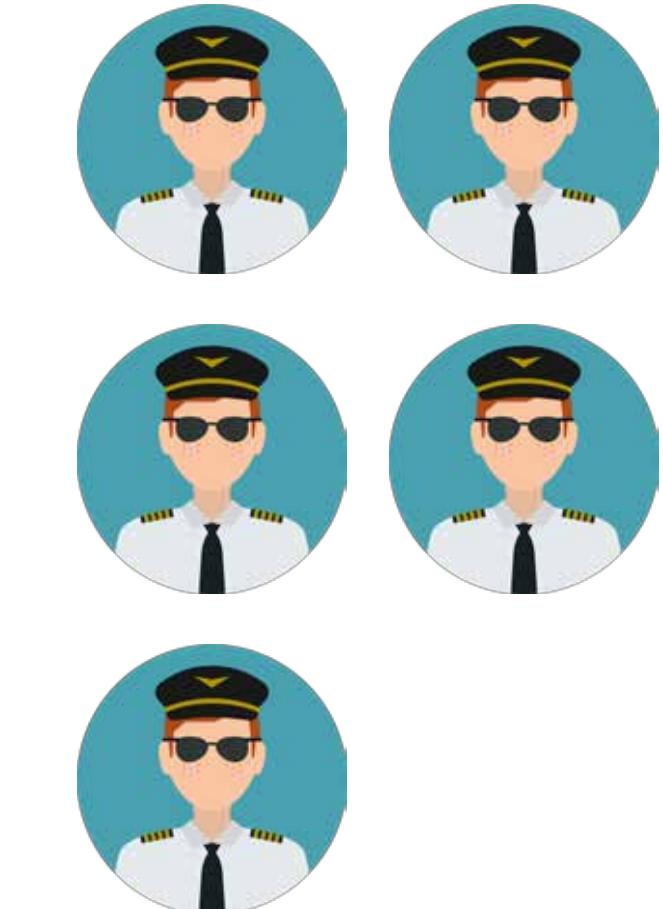
Estudios 1501 y 1502	Estudio 1504
<p>Los participantes recibirán de manera aleatoria (azar):</p> <p>ZX008 durante un máximo de 16 semanas</p> <p>O</p> <p>Un placebo (un medicamento "simulado") durante un máximo de 16 semanas</p> <p>Los participantes tendrán el doble de</p>	<p>Los participantes recibirán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

SEIZURES



Crear sistemas que nos
ayuden a tomar
decisiones

La cabina de un Lockheed Constellation



La cabina de un Airbus 350





dreamstime



Hospital Universitario
Puerto de la Cruz
Santander

OXYGENO
TAKEN BY



**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

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29

foundation
twenty-
nine