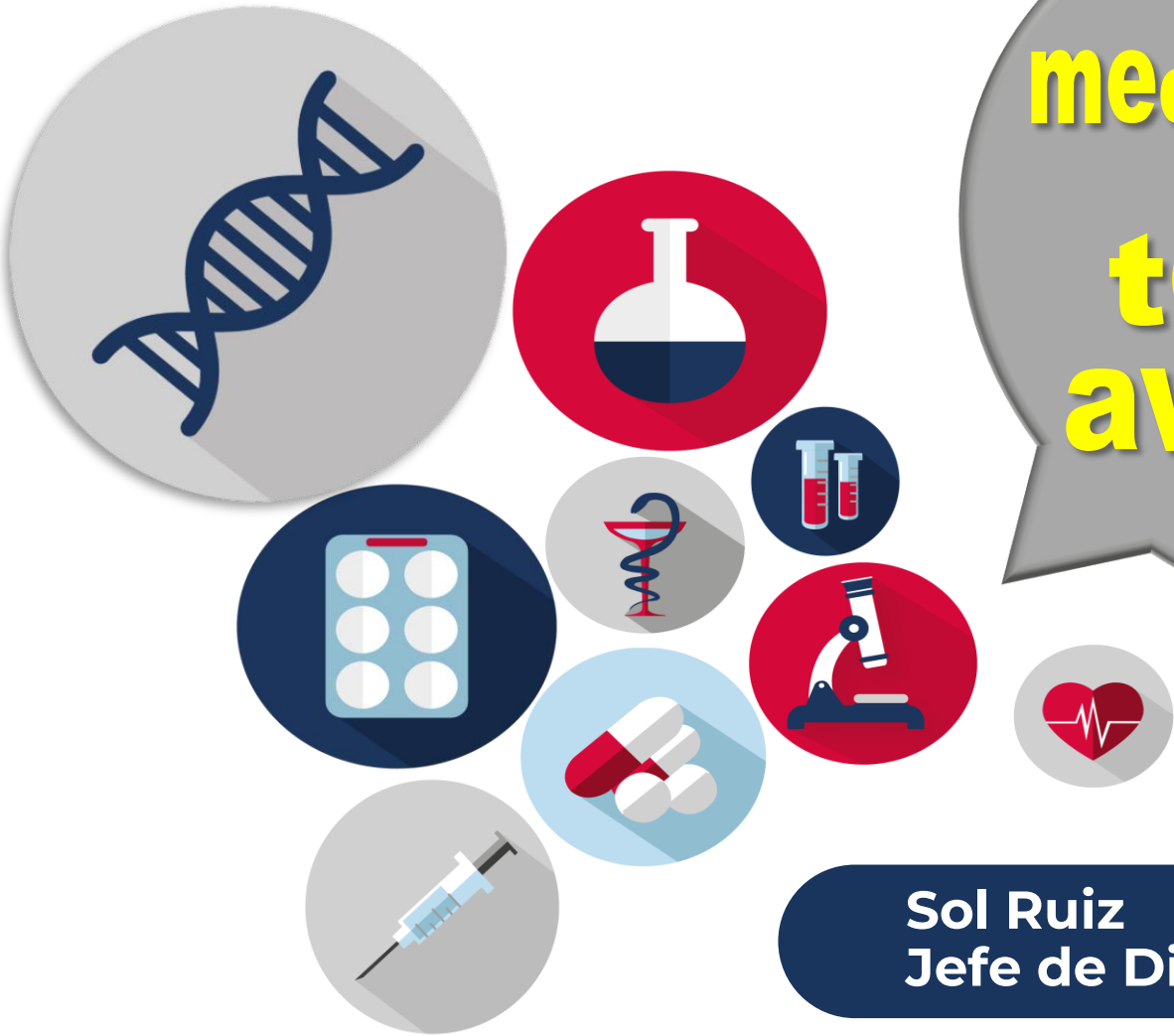
Curso de Derecho Farmacéutico - CEFI



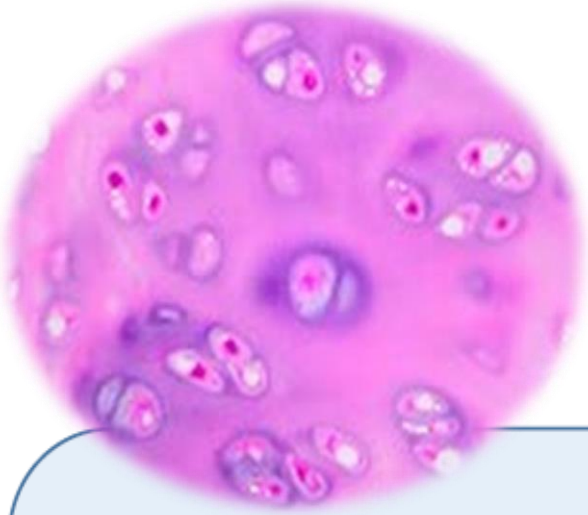
medicamentos de terapia avanzada

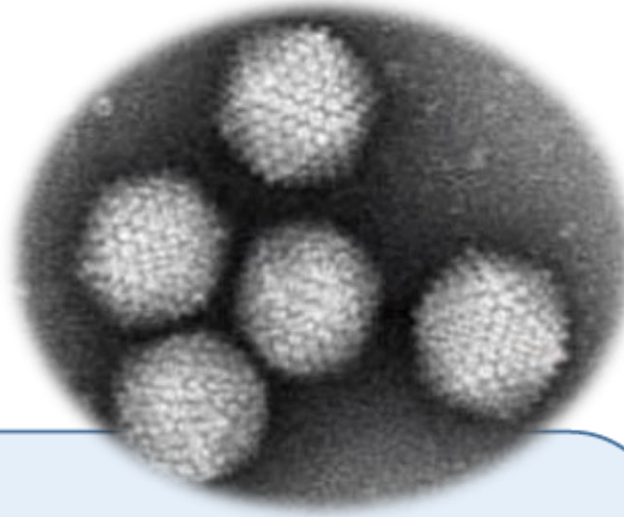
Sol Ruiz
Jefe de División – Biológicos y TA

AGENCIA ESPAÑOLA DE MEDICAMENTOS Y PRODUCTOS SANITARIOS

MINISTERIO DE SANIDAD

agencia española de medicamentos y productos sanitarios





Medicamentos de terapia celular somática

Medicamentos de terapia génica

Medicamentos de terapia avanzada

Medicamentos de ingeniería tisular

Medicamentos combinados de terapia avanzada

Regulation (EC) No 1394/2007

Directive 2009/120/EC

Principio activo:

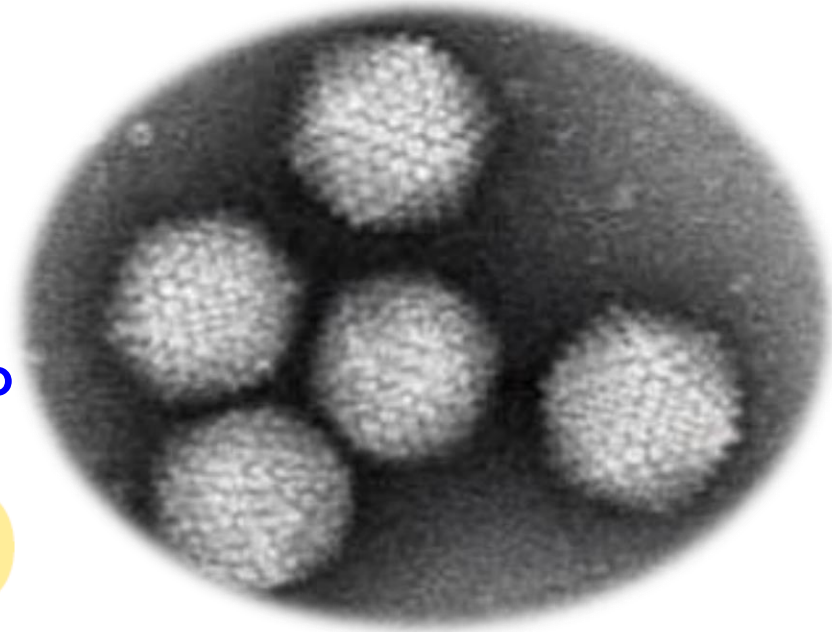
Ácido nucleico recombinante

+ vector viral

- medicamento biológico

Objetivo:

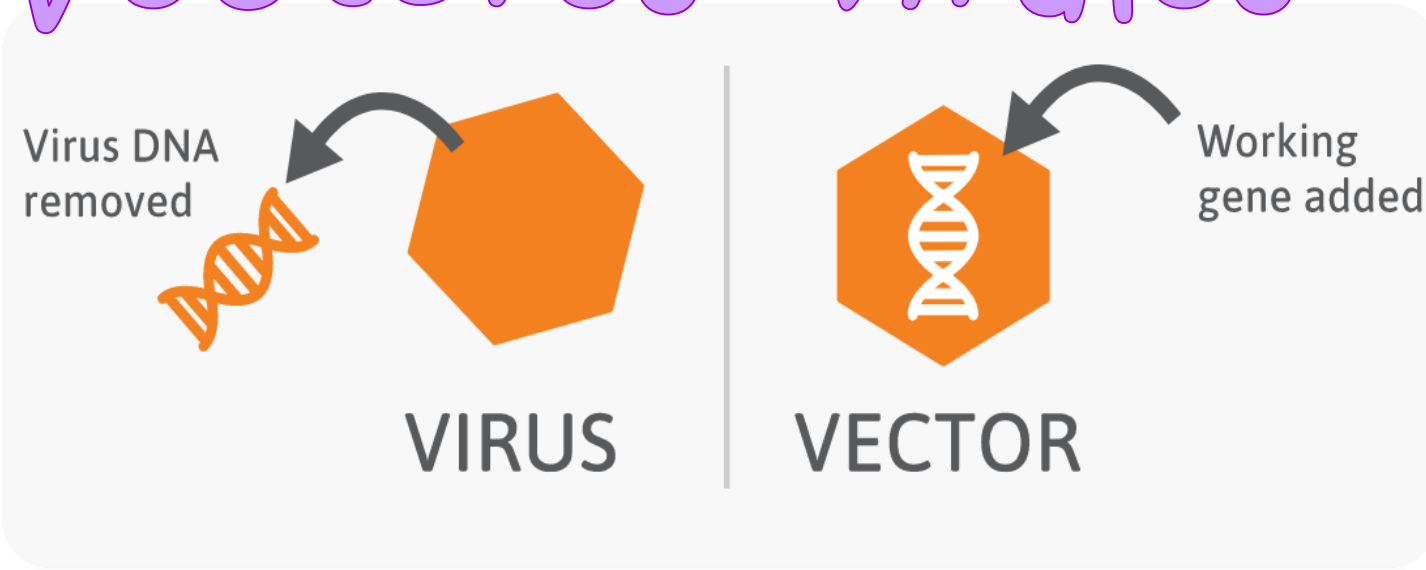
Regular, reparar, sustituir, añadir o eliminar una secuencia génica



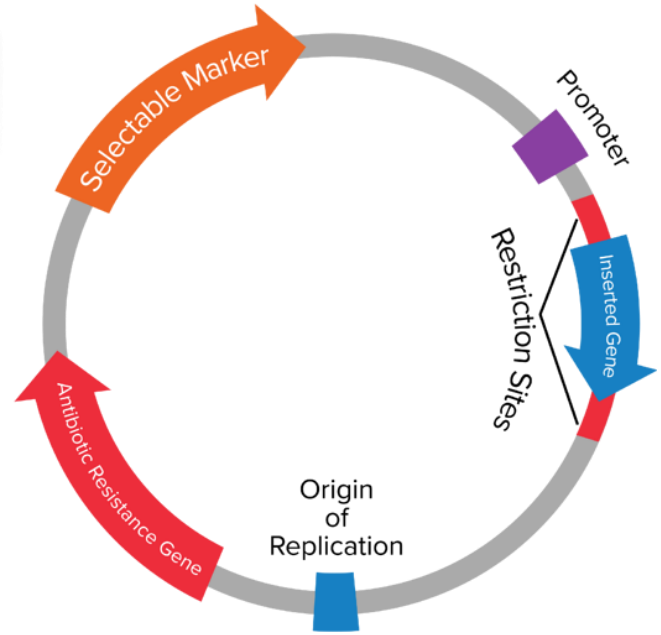
Medicamentos de terapia génica

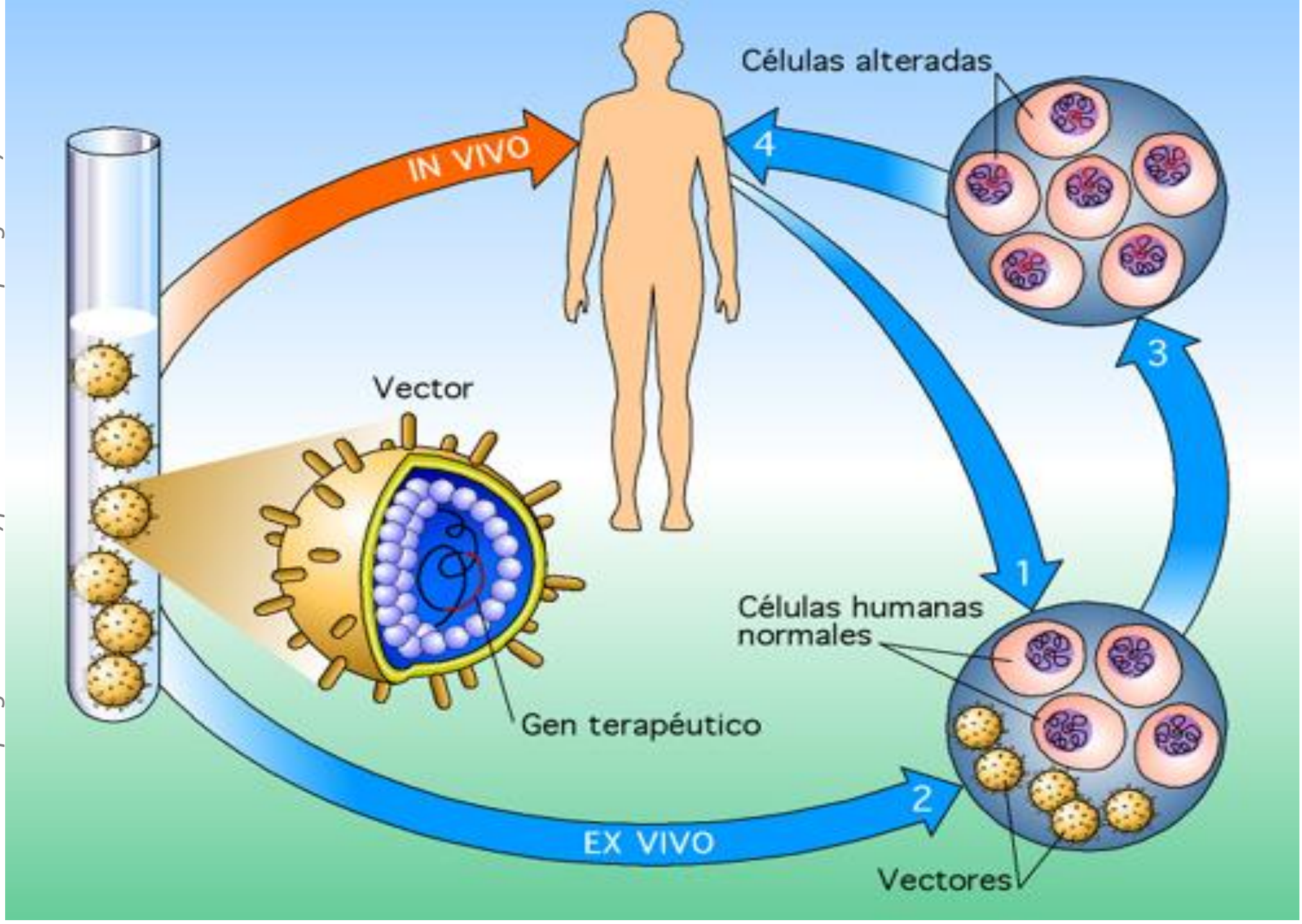
Directive 2009/120/EC

Vectores Virales



Vectores no Virales



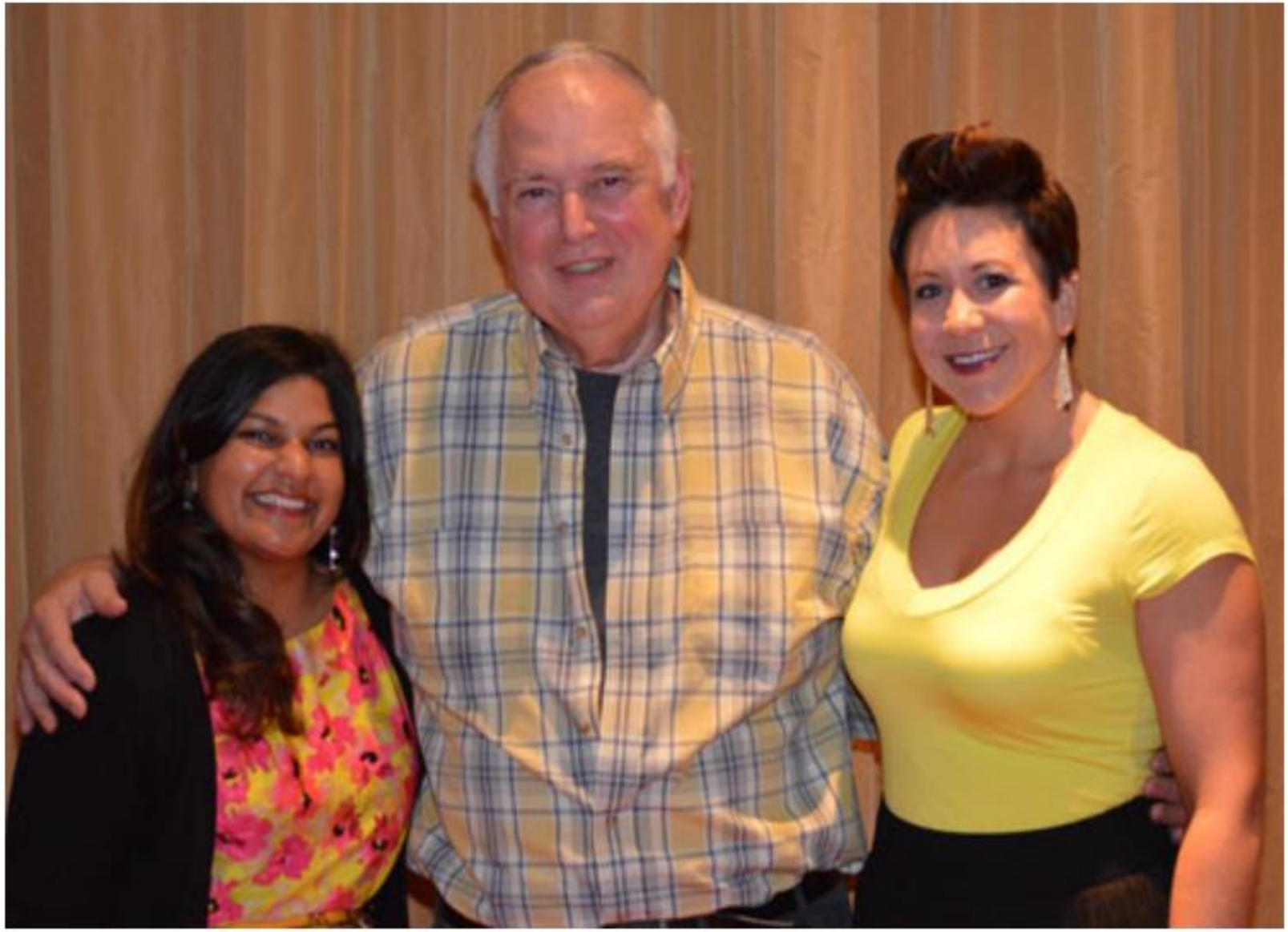


terapiagenica.science/proceso-de-la-terapia-genica/

primaryimmune.org/first-gene-therapy-patients-attend-conference

Cynthia and Ashanthi in 1992 with the pioneer physicians of gene therapy: (from left) French Anderson, MD; Michael Blaese, MD; and Kenneth Culver.

primaryimmune.org/first-gene-therapy-patients-attend-conference



R. Michael Blaese, MD with Ashanthi DeSilva (left) and Cindy Kisik at the IDF 2013 National Conference, June 29.

The New England Journal of Medicine

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VOLUME 346 APRIL 18, 2002 NUMBER 16

SUSTAINED CORRECTION OF X-LINKED SEVERE COMBINED IMMUNODEFICIENCY BY EX VIVO GENE THERAPY

SALIMA HACEIN-BEY-ABINA, PH.D., FRANÇOISE LE DEIST, M.D., PH.D., FRÉDÉRIQUE CARLIER, B.S., CÉCILE BOUNEAUD, PH.D., CHRISTOPHE HUE, B.S., JEAN-PIERRE DE VILLARTAY, PH.D., ADRIAN J. THRASHER, M.D., PH.D., NICOLAS WULFFRAAT, M.D., RICARDO SORENSEN, M.D., SOPHIE DUPUIS-GIROD, M.D., ALAIN FISCHER, M.D., PH.D., AND MARINA CAVAZZANA-CALVO, M.D., PH.D.

ABSTRACT

Background X-linked severe combined immunodeficiency due to a mutation in the gene encoding the common γ (γc) chain is a lethal condition that can be cured by allogeneic stem-cell transplantation. We investigated whether infusion of autologous hematopoietic stem cells that had been transduced in vitro with the *γc* gene can restore the immune system in patients with severe combined immunodeficiency.

DEFICIENCY of the common γ (γc) chain, an X-linked disorder, causes the most frequent form of severe combined immunodeficiency disease.[1,2] The γc chain is an essential component of five cytokine receptors, all of which are necessary for the development of T cells and natural killer cells. Without the γc chain, there is a complete absence of mature T and natural killer cells, whereas B cells are usually present in normal

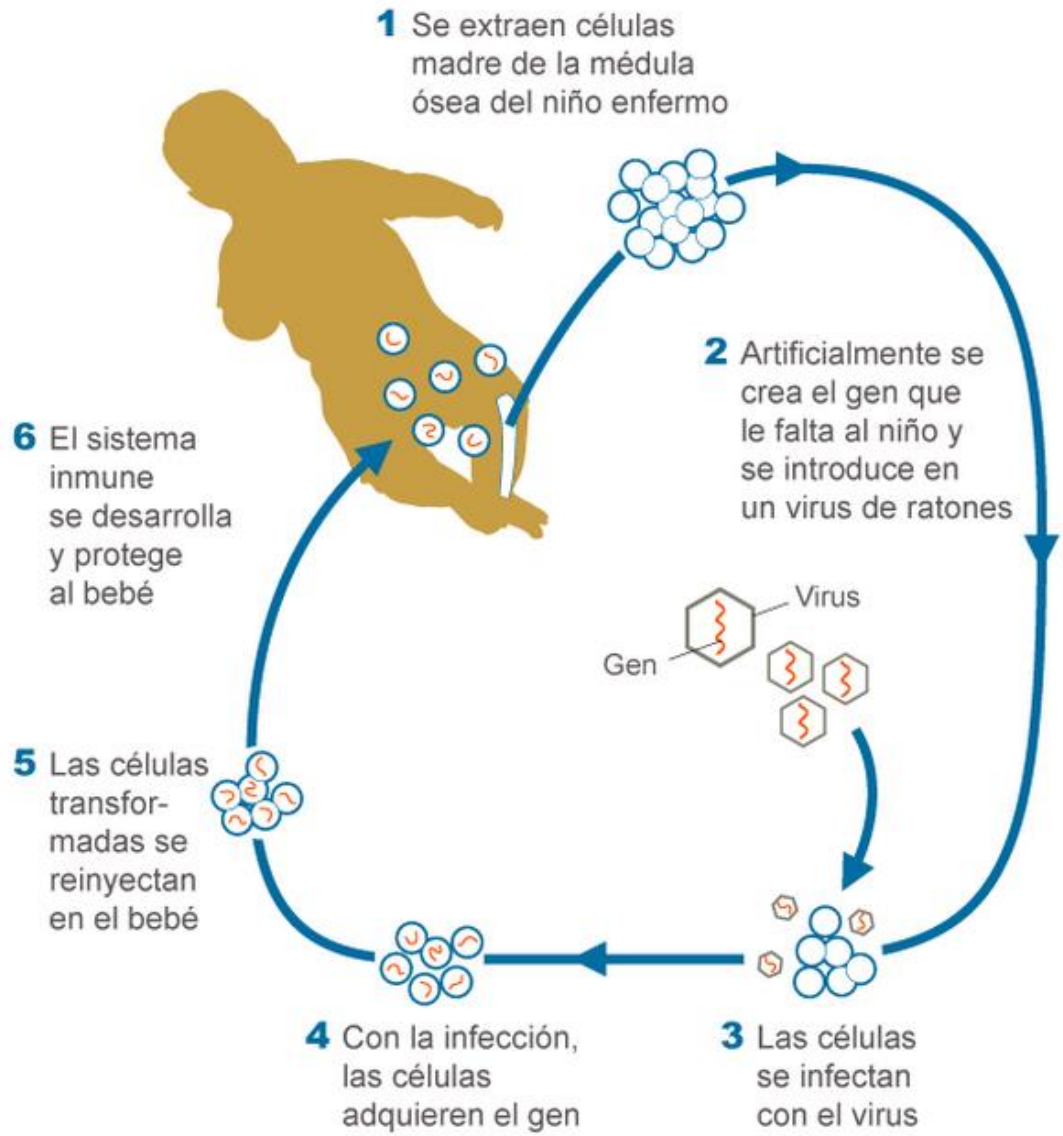
El niño David Vetter, inspirador de la película 'El chico de la burbuja de plástico' (1976) / BCM

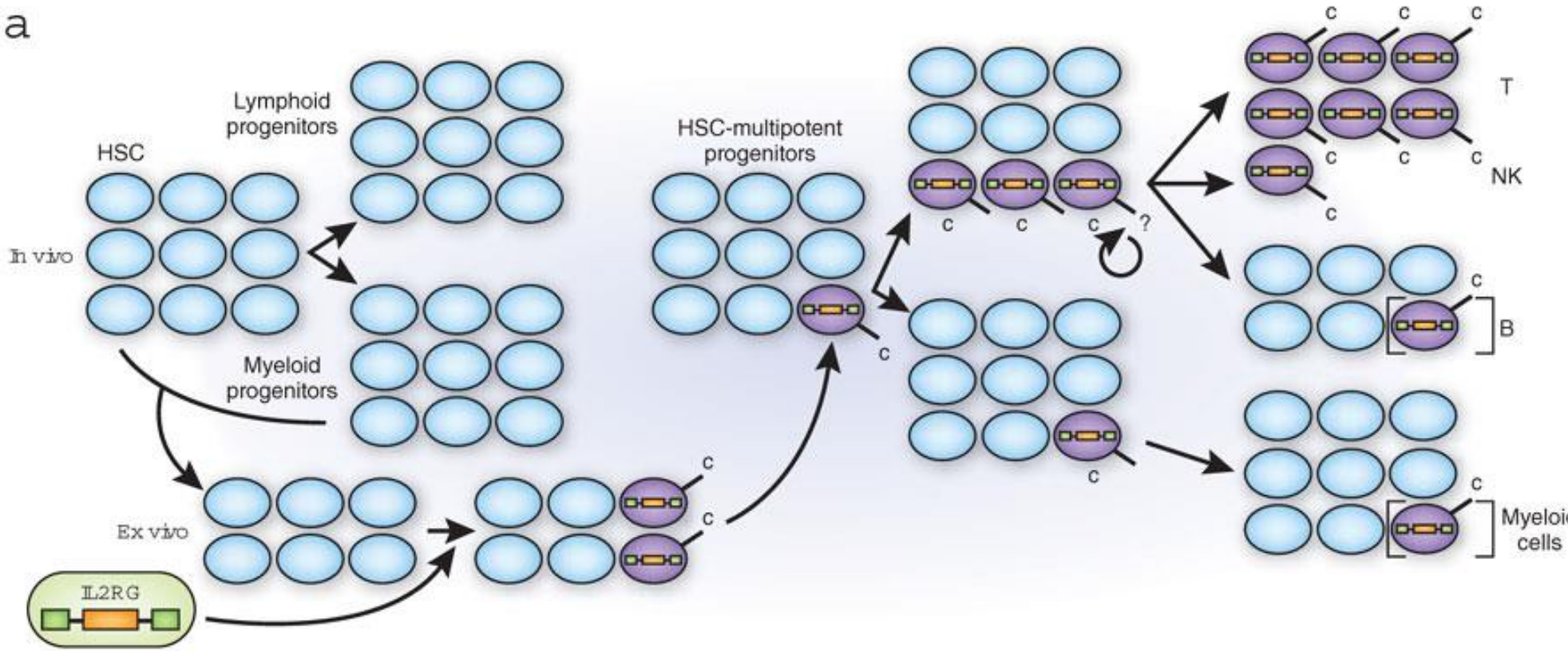
elpais.com/elpais/2014/10/15/ciencia/1413372486_108083.html

TERAPIA GÉNICA PARA 'NIÑOS BURBUJA'

La enfermedad se produce porque un gen implicado en la producción de glóbulos blancos (las defensas del organismo) es defectuoso.

elpais.com/elpais/2014/10/15/ciencia/1413372486_108083.html





Nature Immunology (2010) **volume 11**, 457–460

The NEW ENGLAND JOURNAL of MEDICINE

ESTABLISHED IN 1812 JANUARY 29, 2009 VOL. 360 NO. 5

Gene Therapy for Immunodeficiency Due to Adenosine Deaminase Deficiency

Alessandro Aiuti, M.D., Ph.D., Federica Cattaneo, M.D., Stefania Galimberti, Ph.D., Ulrike Benninghoff, M.D., Barbara Cassani, Ph.D., Luciano Callegaro, R.N., Samantha Scaramuzza, Ph.D., Grazia Andolfi, Massimiliano Mirolo, B.Sc., Immacolata Brigida, B.Sc., Antonella Tabucchi, Ph.D., Filippo Carlucci, Ph.D., Martha Eibl, M.D., Memet Aker, M.D., Shimon Slavin, M.D., Hamoud Al-Mousa, M.D., Abdulaziz Al Ghonaium, M.D., Alina Ferster, M.D., Andrea Duppenthaler, M.D., Luigi Notarangelo, M.D., Uwe Wintergerst, M.D., Rebecca H. Buckley, M.D., Marco Bregni, M.D., Sarah Marktel, M.D., Maria Grazia Valsecchi, Ph.D., Paolo Rossi, M.D., Fabio Ciceri, M.D., Roberto Miniero, M.D., Claudio Bordignon, M.D., and Maria-Grazia Roncarolo, M.D.

BACKGROUND

We investigated the long-term outcome of gene therapy for severe combined immunodeficiency (SCID) due to the lack of adenosine deaminase (ADA), a fatal disorder of purine metabolism and immunodeficiency.

METHODS

We infused autologous CD34+ bone marrow cells transduced with a retroviral vector containing the *ADA* gene into 10 children with SCID due to ADA deficiency who lacked an HLA-identical sibling donor, after nonmyeloablative conditioning with busulfan. Enzyme-replacement therapy was not given after infusion of the cells.

RESULTS

All patients are alive after a median follow-up of 4.0 years (range, 1.8 to 8.0). Transduced hematopoietic stem cells have stably engrafted and differentiated into myeloid cells containing ADA (mean range at 1 year in bone marrow lineages, 3.5 to 8.9%) and lymphoid cells (mean range in peripheral blood, 52.4 to 88.0%). Eight patients do not require enzyme-replacement therapy, their blood cells continue to express ADA, and they have no signs of defective detoxification of purine metabolites. Nine patients had immune reconstitution with increases in T-cell counts (median count at 3 years, 1.07×10^9 per liter) and normalization of T-cell function. In the five patients in whom intravenous immune globulin replacement was discontinued, antigen-specific antibody responses were elicited after exposure to vaccines or viral antigens. Effective protection against infections and improvement in physical development made a normal lifestyle possible. Serious adverse events included prolonged neutropenia (in two patients), hypertension (in one), central-venous-catheter–related infections (in two), Epstein–Barr virus reactivation (in one), and autoimmune hepatitis (in one).

CONCLUSIONS

Gene therapy, combined with reduced-intensity conditioning, is a safe and effective treatment for SCID in patients with ADA deficiency. (ClinicalTrials.gov numbers, NCT00598481 and NCT00599781.)

From the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET) (A.A., F.C., U.B., B.C., L.C., S. Scaramuzza, G.A., M.M., I.B., S.M., M.-G.R.), University of Milan-Bicocca (S.G., M.G.V.), Ospedale San Giuseppe (M.B.), San Raffaele Scientific Institute (F.C.), Università Vita–Salute San Raffaele (C.B., M.-G.R.), and MolMed (C.B.) — all in Milan; Tor Vergata University (A.A., P.R.) and Children's Hospital Bambino Gesù (P.R.) — both in Rome; University of Siena, Siena (A.T., F.C.); and University of Turin, Turin (R.M.) — all in Italy; Immunologische Tagesklinik, Vienna, Austria (M.E.); Hadassah University Hospital, Jerusalem, Israel (M.A., S. Slavin); King Faisal Specialist Hospital and Research Center, Riyadh, Saudi Arabia (H.A.-M., A.A.G.); Hôpital Universitaire des Enfants Reine Fabiola–Université Libre de Bruxelles, Brussels (A.F.); University Children's Hospital, Bern, Switzerland (A.D.); Children's Hospital, Harvard Medical School, Boston (L.N.); Universitäts-Kinderklinik München, Munich, Germany (U.W.); and Duke University Medical Center, Durham, NC (R.H.B.). Address reprint requests to Dr. Roncarolo at HSR-TIGET, Via Olgettina 58, 20132 Milan, Italy, or at m.roncarolo@hsr.it.

N Engl J Med 2009;360:447-58.

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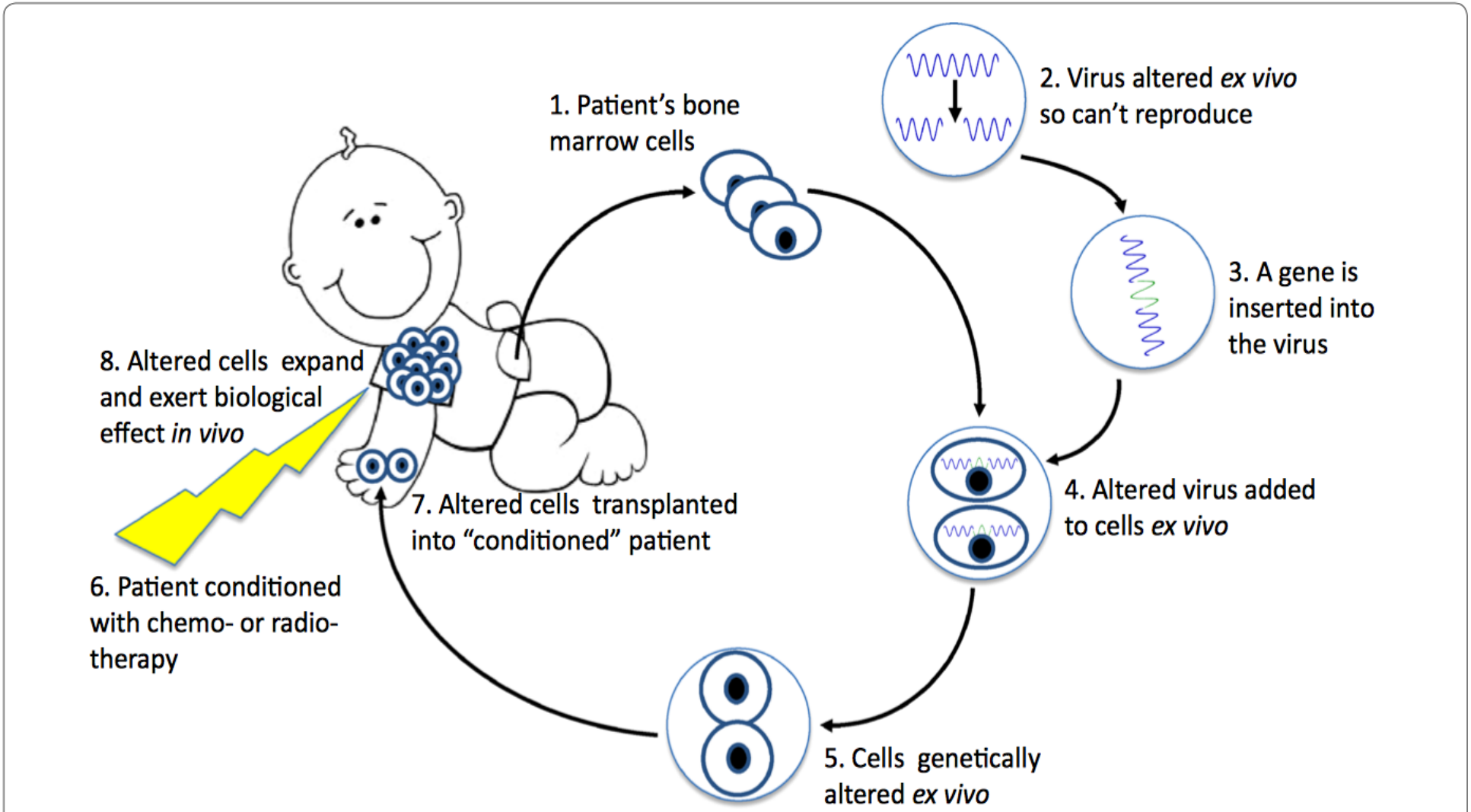
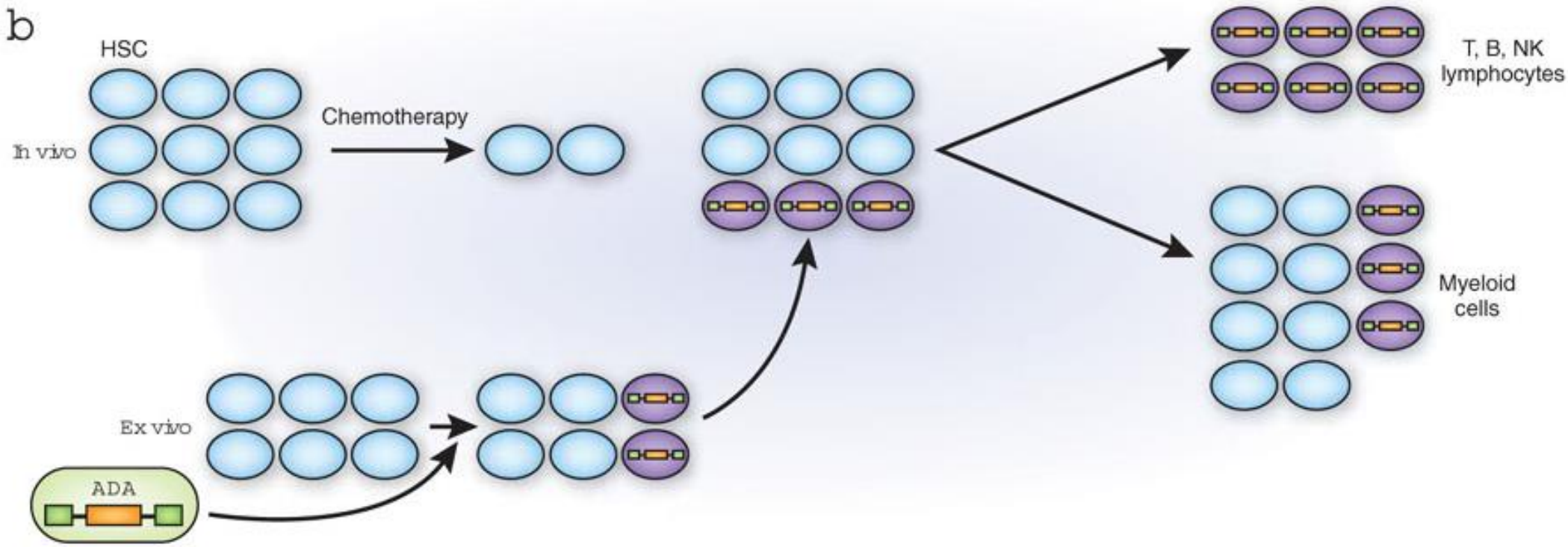


Fig. 1 Ex vivo gene therapy. Patient's cells are collected from bone marrow, peripheral blood or umbilical cord blood (*1*). A virus is altered ex vivo to increase safety and efficacy of gene delivery (*2*). A gene is inserted into the altered virus ex vivo (*3*). The altered virus containing the gene is added to the patient's cells ex vivo (*4*). The cells are genetically altered ex vivo (*5*). The patient is treated with chemotherapy or radiotherapy (*6*). The genetically altered cells are transplanted into the conditioned patient (*7*). The genetically altered cells expand in the patient and exert their biological effects (*8*)

aacijournal.biomedcentral.com/articles/10.1186/s13223-017-0184-y



Nature Immunology (2010) **volume 11**, 457–460

1 April 2016
EMA/CHMP/230486/2016
Press office

Strimvelis

Press release

New gene therapy for the treatment of children with ultra-rare immune disorder recommended for approval

Orphan-designated Strimvelis to offer treatment option for patients with ADA-SCID who have no suitable stem cell donor

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for a new gene therapy for the treatment of patients with adenosine-deaminase-deficient severe combined immunodeficiency (ADA-SCID), who have no matching donor for a stem cell transplant. Children born with this disorder have virtually no immunity to fight off everyday bacterial, fungal or viral infections.

ADA-SCID is an ultra-rare immune disorder, caused by a faulty gene inherited from both parents that stops the production of adenosine deaminase. Without this enzyme, the body is unable to break down a toxic substance called deoxyadenosine. The toxin builds up and destroys infection-fighting lymphocytes. Children born with ADA-SCID are severely impaired in their ability to fight infections. The disorder can also lead to various non-immunological health problems, including a failure to grow and develop normally, hearing loss and liver and kidney problems.

Symptoms normally appear in the first six months of life. The disease is usually fatal in the first two years of life, unless the function of the immune system can be restored.

Strimvelis

The effects of Strimvelis were studied in a pivotal clinical trial involving 12 patients. All of the patients included in this trial are still alive, with an average follow-up period of 7 years. The most common side effects observed in this study include pyrexia (fever), increased hepatic enzyme levels, autoimmune reactions, such as anaemia, neutropenia, and autoimmune haemolytic anaemia, aplastic anaemia and thrombocytopenia. This study was carried out in accordance with a Paediatric Investigation Plan (PIP), which was agreed by the Agency's Paediatric Committee. To ensure close long-term follow-up, the applicant for Strimvelis, is required to enrol all patients who receive the medicines, in a registry to monitor and report its long-term effects.

www.ema.europa.eu/en/medicines/human/EPAR/strimvelis

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 March 2019
EMA/196197/2019
Media and Public Relations

Press release

New gene therapy to treat rare inherited blood condition

EMA has recommended granting a marketing authorisation in the European Union for a genetically modified product for beta-thalassaemia, a rare inherited blood condition that causes severe anaemia. Zynteglo is intended for adult and adolescent patients 12 years and older who need regular blood transfusions to manage their disease and have no matching donor for a stem cell transplant.

In the two main studies to demonstrate the effects of Zynteglo it was shown that the majority of patients who do not have a β0/β0 genotype treated with Zynteglo no longer needed regular blood transfusions.

The most common side effects were thrombocytopenia (low blood platelet count), abdominal pain, non-cardiac chest pain, pain in the extremities, dyspnoea and hot flush.

Since Zynteglo addresses an unmet medical need, it benefited from PRIME, EMA's platform for early and enhanced dialogue with developers of promising new medicines. This interaction led to a more robust application package to demonstrate the medicine's benefits and risks, which allowed accelerated assessment of Zynteglo in 150 days, the fastest advanced-therapy medicinal product (ATMP) review time to date.

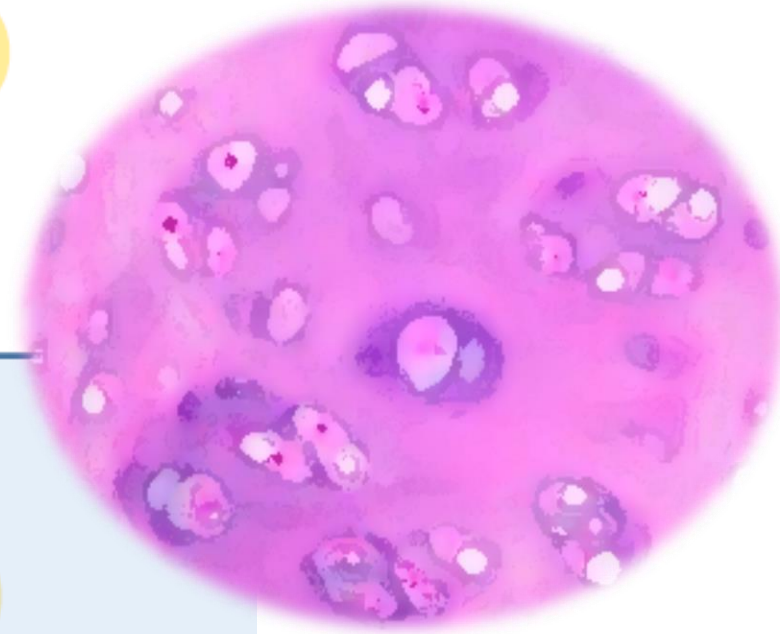
www.ema.europa.eu/en/medicines/human/EPAR/zynteglo

Principio activo:

Células o tejidos

Objetivo:

Prevenir, tratar o diagnosticar una enfermedad mediante la acción farmacológica, inmunológica o metabólica de las células o tejidos



Medicamentos de terapia celular somática

Directive 2009/120/EC

terapia avanzada

Medicamentos de ingeniería tisular

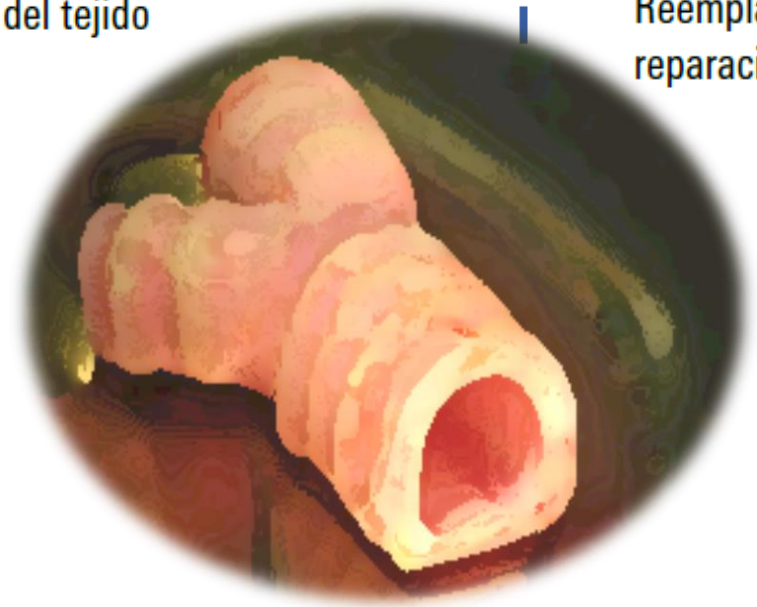
Principio activo:
Células o tejidos

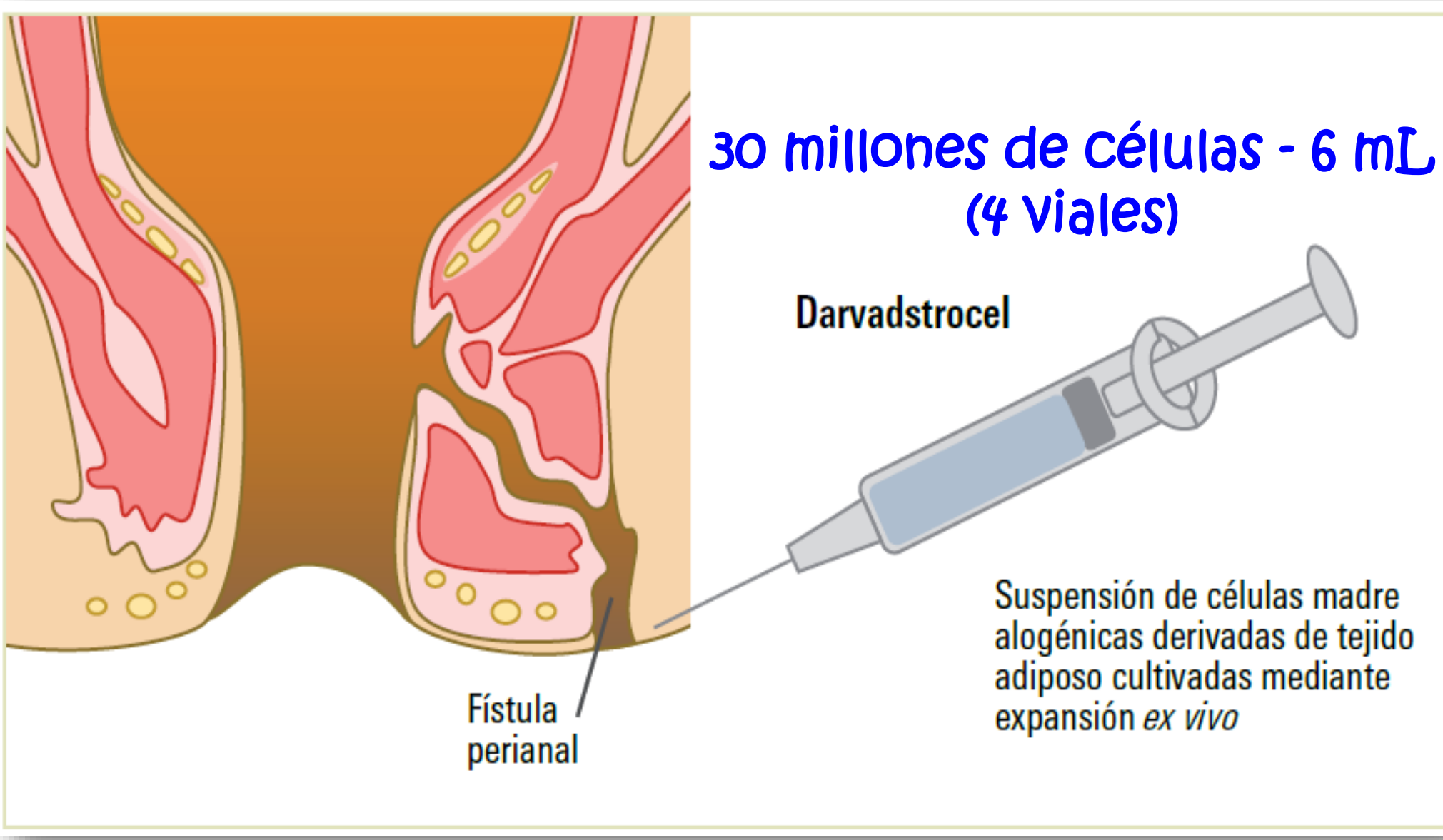
Objetivo:
Reemplazo, sustitución o reparación estructural del tejido

Medicamentos combinados de terapia avanzada

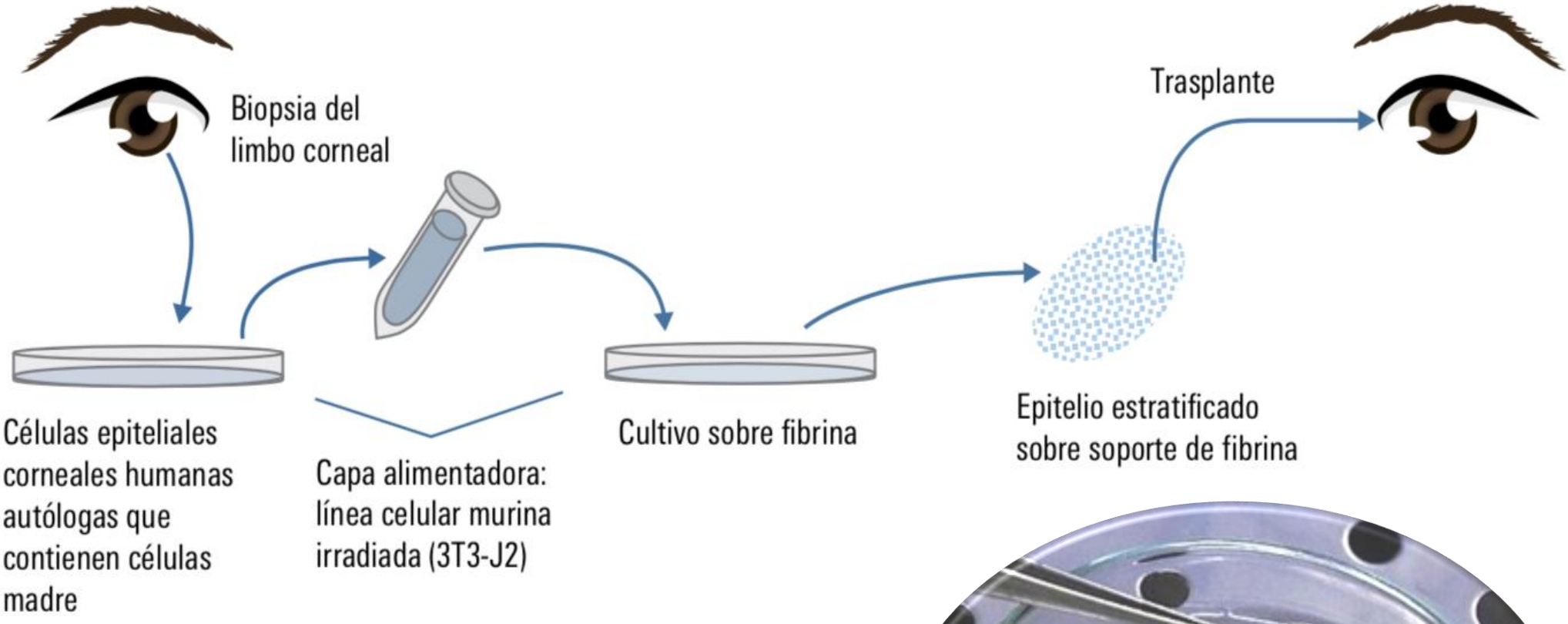
Principio activo:
Células o tejidos
+ producto sanitario

Objetivo:
Reemplazo, sustitución o reparación estructural del tejido





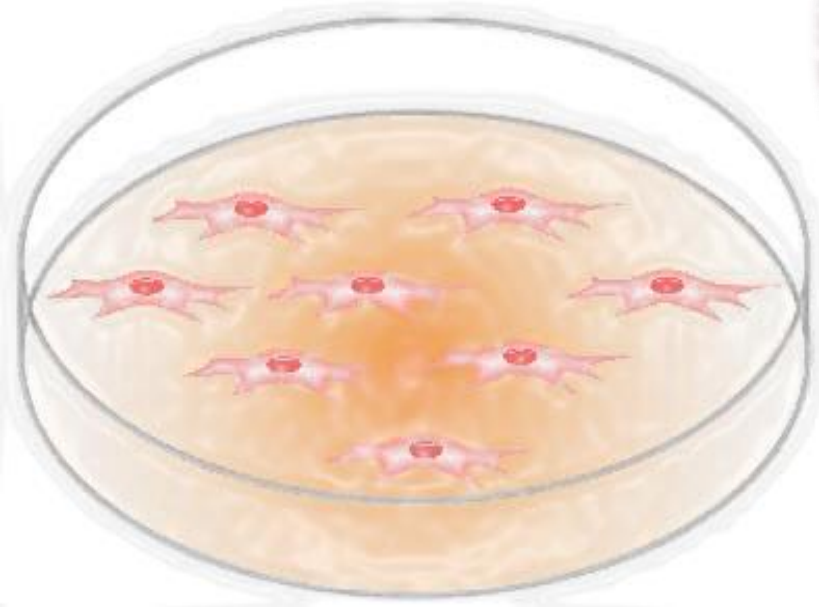
S Ruiz (2018). *Terapias avanzadas en patologías musculares, articulares e inmunológicas* en *Trastornos osteoarticulares, musculares e inmunológicos*. Consejo General de Colegios Oficiales de Farmacéuticos (ed.)

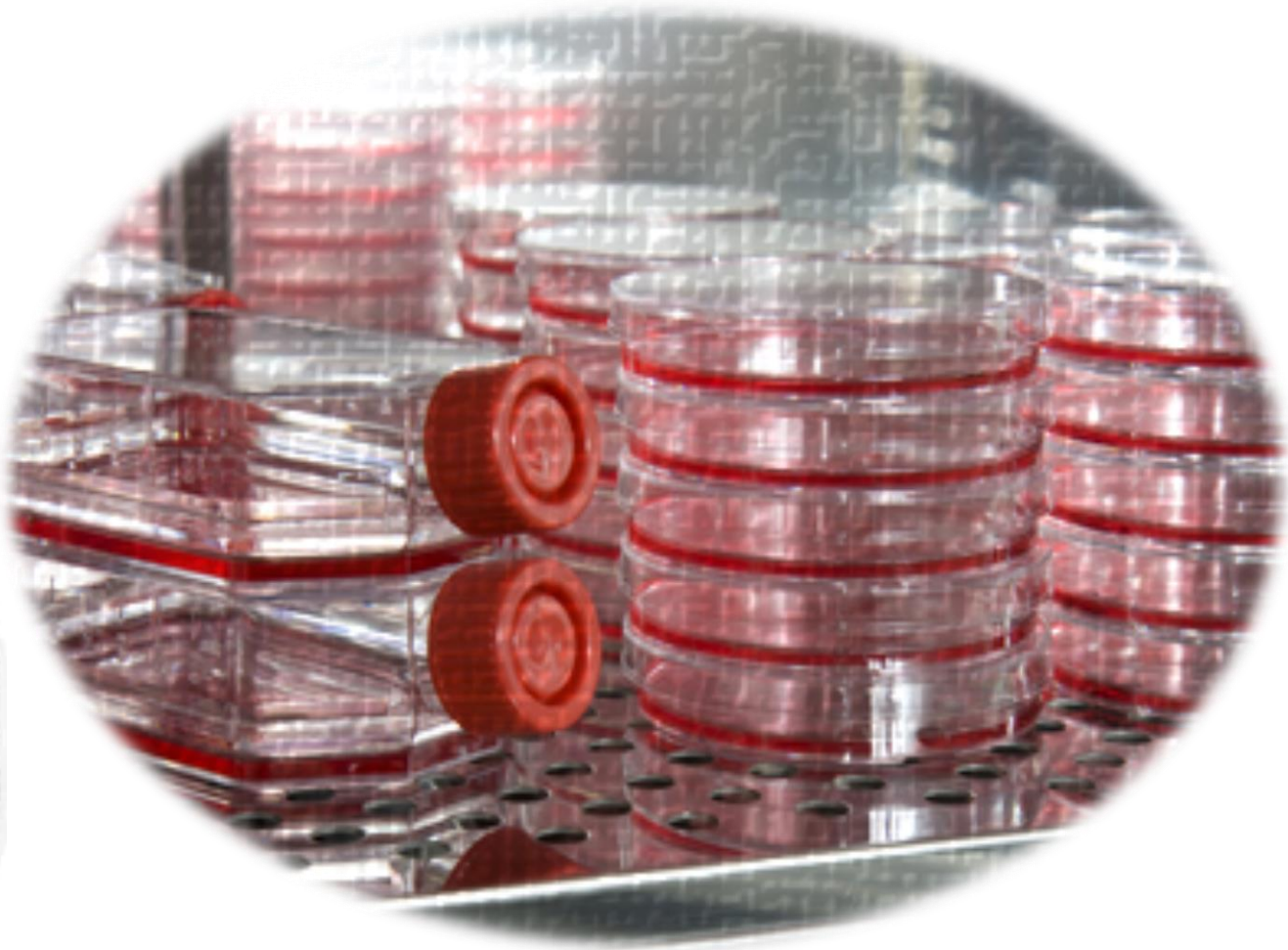


Holoclar® se utiliza para el tratamiento de pacientes adultos con deficiencia de células madre limbares (LSCD, *limbal stem cell deficiency*) de moderada a grave debido a quemaduras oculares por agentes físicos o químicos. Está destinado a ser trasplantado en

S Ruiz (2018). *Terapias avanzadas en patologías musculares, articulares e inmunológicas* en *Trastornos osteoarticulares, musculares e inmunológicos*. Consejo General de Colegios Oficiales de Farmacéuticos (ed.)

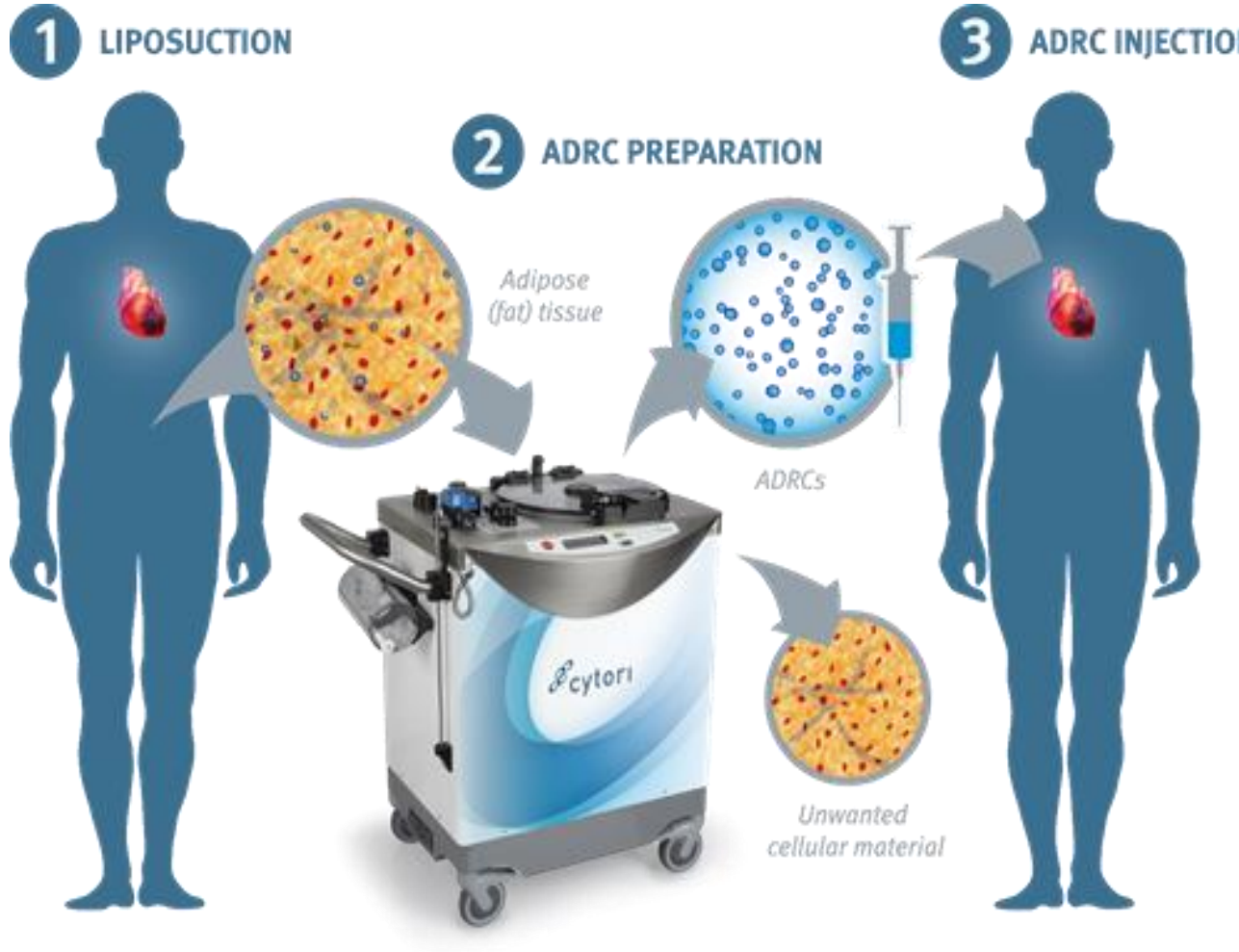
medicamento de terapia celular somática





- manipulación sustancial

medicamento de terapia celular somática



cardiovascularnews.com/cytori-technology-selected-for-nhlbi-funded-trial-in-lvad-patients

- Cambio de función celular esencial

www.hindawi.com/journals/sci/2018/8031718/

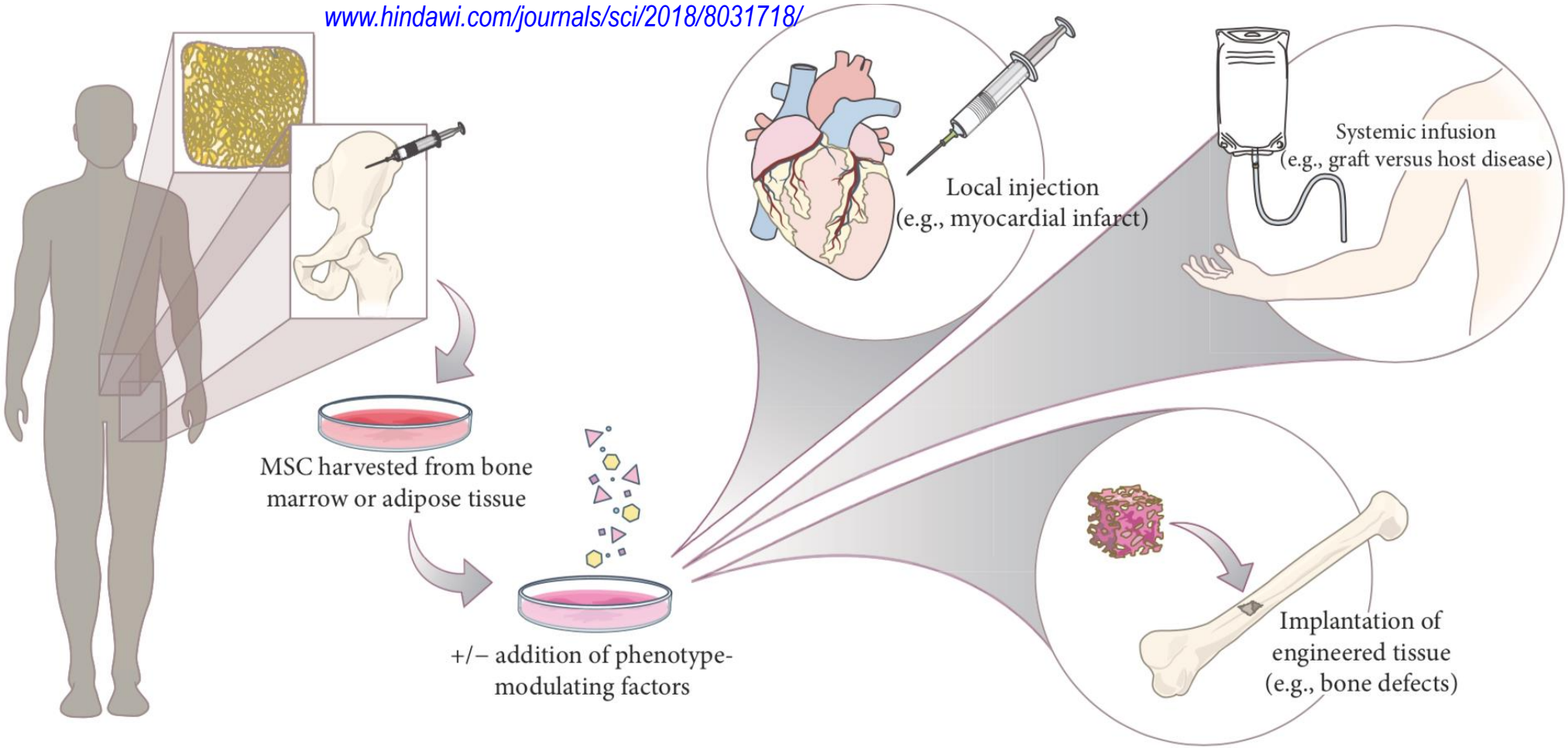


FIGURE 1: Strategies for mesenchymal stromal/stem cell- (MSC-) based therapies. MSCs may be isolated from a number of tissues (e.g., bone marrow, adipose tissue, and umbilical cord) and optionally cultured prior to clinical use. Depending on the specific application, MSC suspensions may then be introduced intravenously or by local injection to achieve the desired therapeutic effects, such as treating autoimmune diseases or stimulating local tissue repair and vascularization, respectively. MSCs may also be utilized for engineering tissues by first promoting their differentiation toward a desired cell type (e.g., osteoblasts, chondrocytes, and adipocytes) prior to being surgically implanted, often along with scaffold material.

- Tratamiento, prevención, diagnóstico

medicamentos de terapia avanzada

- autorización de comercialización
- investigación clínica
- autorización de uso

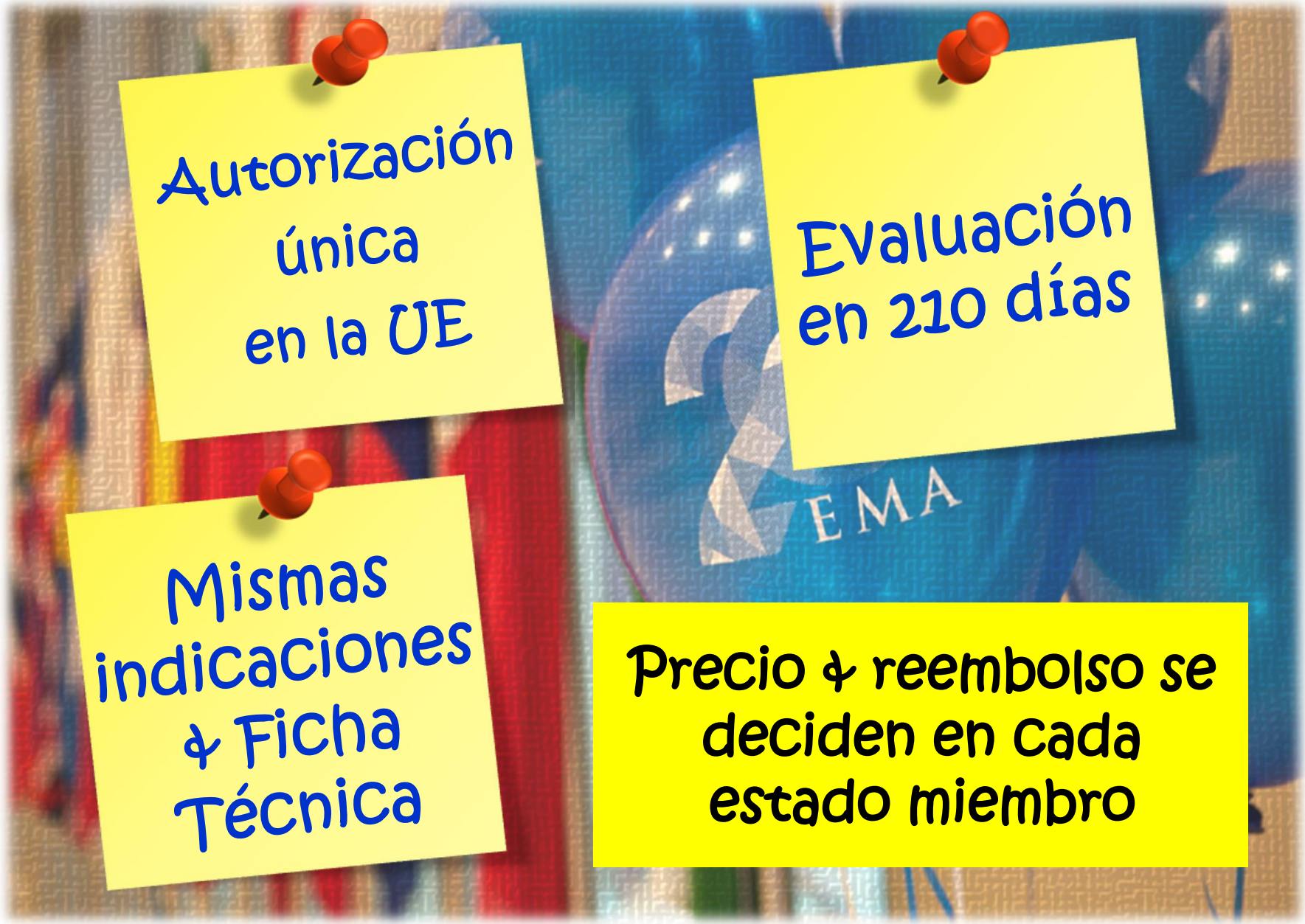
medicamentos de terapia avanzada

autorización de comercialización

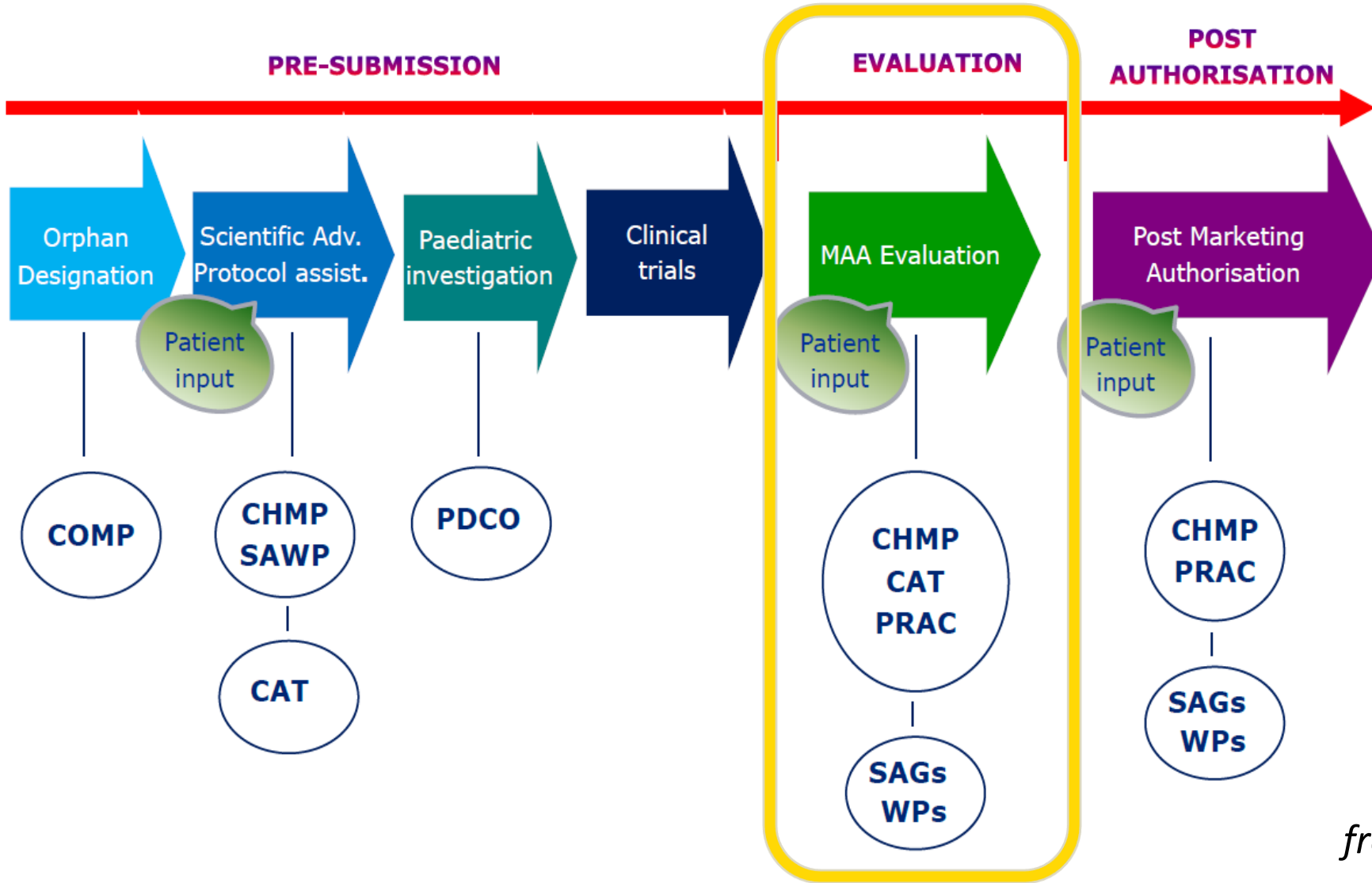
investigación clínica

autorización de uso

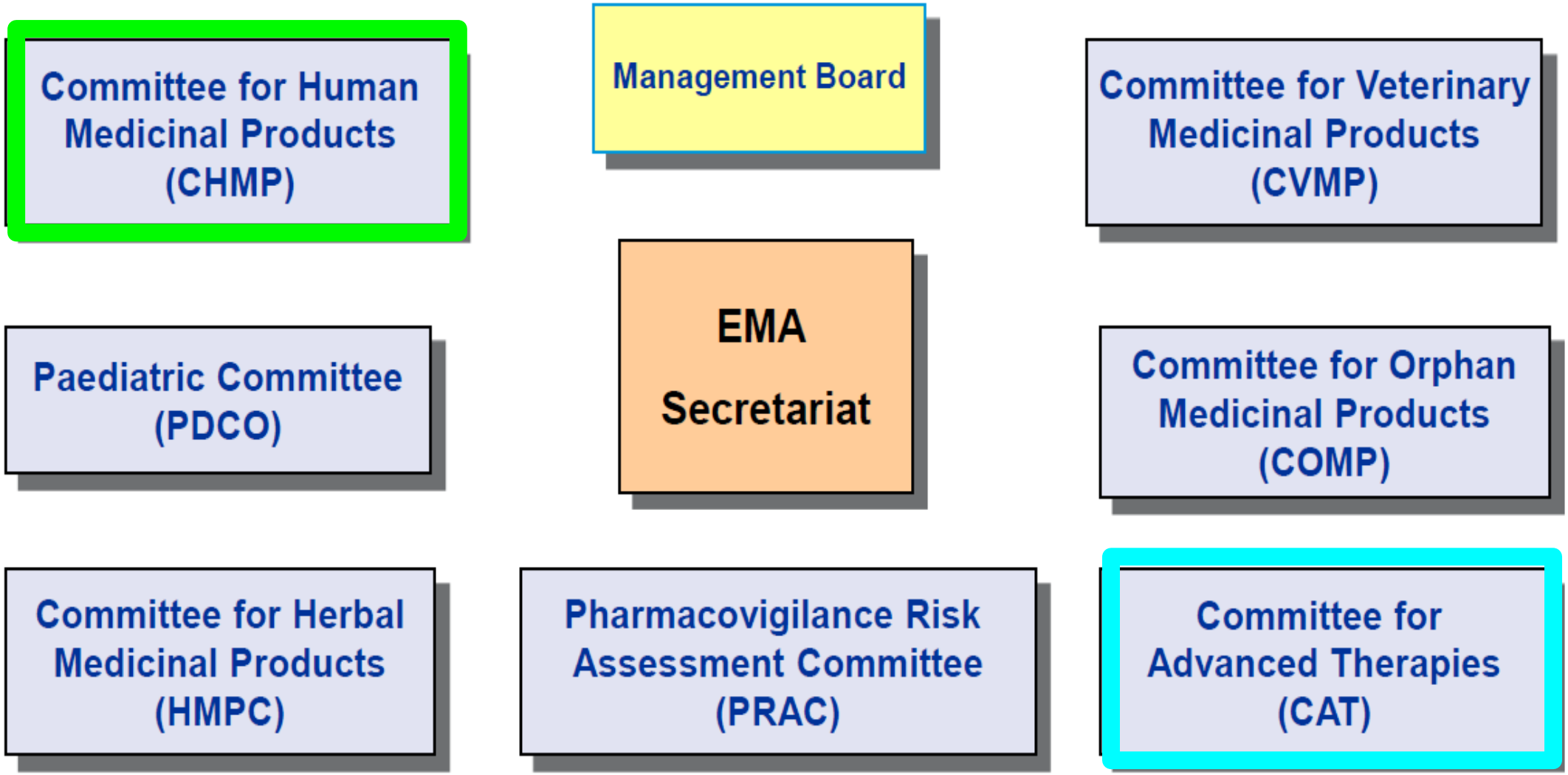
PROCEDIMIENTO CENTRALIZADO



Centralised procedure - product life-cycle



from EMA


Committee for Human Medicinal Products (CHMP)
Management Board
Committee for Veterinary Medicinal Products (CVMP)
Paediatric Committee (PDCO)
EMA Secretariat
Committee for Orphan Medicinal Products (COMP)
Committee for Herbal Medicinal Products (HMPC)
Pharmacovigilance Risk Assessment Committee (PRAC)
Committee for Advanced Therapies (CAT)

from EMA

Meeting highlights from the Committee for Medicinal Products for Human Use (CHMP) 22-25 February 2021

Share

News 26/02/2021

Six new medicines recommended for approval

EMA's human medicines committee (CHMP) recommended six medicines for approval at its February 2021 meeting.

The Committee recommended granting a marketing authorisation for **Evrysdi*** (risdiplam), the first treatment that can be given orally to patients with certain types of spinal muscular atrophy, a rare and often fatal genetic disease that causes muscle weakness and progressive loss of movement. Since Evrysdi addresses an unmet medical need, it benefited from support through the PRIME scheme, EMA's platform for early and enhanced dialogue with developers of promising new medicines. Evrysdi was reviewed under EMA's accelerated assessment programme. For more information, see the press release in the grid below.

The CHMP recommended granting a conditional marketing authorisation for **Jemperli** (dostarlimab) for the treatment of certain types of recurrent or advanced endometrial cancer.

medicamentos autorizados de terapia avanzada

07/2009	**CHONDROCELECT**	Autologous chondrocytes
07/2012	**GLYBERA**	AAV-LPL gene
04/2013	**MACI**	Matrix-induced autologous chondrocyte implantation
06/2013	**PROVENGE**	Autologous PBMC activated with PAP-GM-CSF
12/2014	**HOLOCLAR**	Autologous human corneal epithelial cells
10/2015	**IMLYGIC**	Oncolytic HSV-1 - GM-CSF
04/2016	**STRIMVELIS**	Autologous CD34+ - RV hu ADA gene
06/2016	**ZALMOXIS**	Allogeneic T cells – RV HSV-TK
05/2017	**SPHEROX**	Spheroids autologous matrix-associated chondrocytes
12/2017	**ALOFISEL**	Allogeneic expanded adipose stem cells
06/2018	**KYMRIAH**	Autologous T cells - LV anti-CD19 CAR
06/2018	**YESCARTA**	Autologous T cells - RV anti-CD19 CD28/CD3 zeta CAR
09/2018	**LUXTURNA**	AAV2-hu RPE65
05/2019	**ZYNTEGLO**	Autologous CD34+ - LV βA-T87Q -globin gene
03/2020	**ZOLGENSMA**	AAV9-SMN1
12/2020	**TECARTUS**	Autologous T cells - RV anti-CD19 CD28/CD3-zeta CAR
12/2020	**LIBMELDY**	Autologous CD34+ cell LV human arylsulfatase A gene

07/2009	CHONDROCELECT	Autologous chondrocytes
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09/2018	LUXTURNA	AAV2-hu RPE65
05/2019	ZYNTEGLO	Autologous CD34+ - LV βA-T87Q -globin gene
03/2020	ZOLGENSMA	AAV9-SMN1
12/2020	TECARTUS	Autologous T cells - RV anti-CD19 CD28/CD3-zeta CAR
12/2020	LIBMELDY	Autologous CD34+ cell LV human arylsulfatase A gene

20 May 2021
EMA/CHMP/273940/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pending EC decision

Skysona

elivaldogene autotemcel

On 20 May 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Skysona,[2] intended for the treatment of early cerebral adrenoleukodystrophy (CALD).

As Skysona is a gene therapy, the CHMP's positive opinion is based on an assessment by the Committee for Advanced Therapies. The applicant for this medicinal product is bluebird bio (Netherlands) B.V.

Skysona will be available as a 2-30 x 10^6 cells/ml dispersion for infusion. The active substance of Skysona is elivaldogene autotemcel, which is made specifically for each patient, using the patient's haematopoietic stem cells. The stem cells are modified in a laboratory to insert a working gene for making human adrenoleukodystrophy protein (ALDP). When the patient is given Skysona, which is made up of these modified cells, the cells start making ALDP, which will then break down the very long chain fatty acids that build-up in patients with CALD.

Skysona provides clinically meaningful benefits by preserving motor function and communication ability, and it improves survival in the early stage of cerebral disease.

The most serious adverse reaction attributed to Skysona was pancytopenia. There are some adverse

medicamentos de terapia avanzada

autorización de comercialización

investigación clínica

autorización de uso

Reglamento (CE) No 1394/2007

(6) El presente Reglamento constituye una *lex specialis* que introduce disposiciones adicionales a las establecidas en la Directiva 2001/83/CE. El ámbito de aplicación del mismo debe ser la reglamentación de los medicamentos de terapia avanzada que estén destinados a ser comercializados en los Estados miembros y estén preparados industrialmente o en cuya fabricación intervenga un proceso industrial, de conformidad con el ámbito de aplicación general de la legislación farmacéutica comunitaria que se establece en el título II de la Directiva 2001/83/CE. Deben excluirse del ámbito del presente Reglamento los medicamentos de terapia avanzada preparados ocasionalmente, de acuerdo con normas de calidad específicas, y empleados en un mismo Estado miembro, en un hospital y bajo la responsabilidad profesional exclusiva de un médico colegiado, con el fin de cumplir una prescripción facultativa individual de un producto hecho a medida destinado a un solo paciente, asegurando, al mismo tiempo, que no se menoscaban las normas comunitarias relativas a la calidad y la seguridad.

Cláusula de exclusión hospitalaria

BOLETÍN OFICIAL DEL ESTADO
Núm. 144 Sábado 14 de junio de 2014 Sec. I. Pág. 45068

I. DISPOSICIONES GENERALES

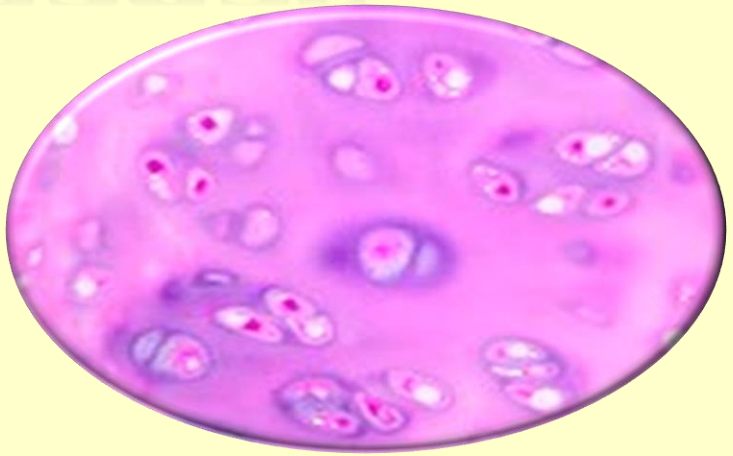
MINISTERIO DE SANIDAD, SERVICIOS SOCIALES E IGUALDAD

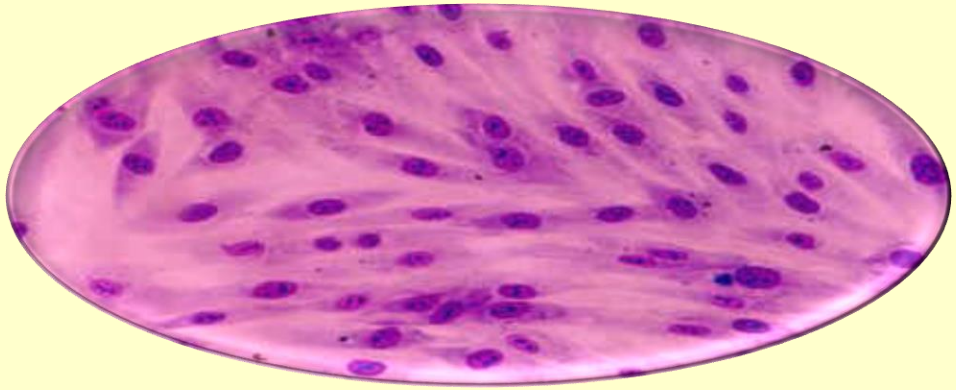
6277 *Real Decreto 477/2014, de 13 de junio, por el que se regula la autorización de medicamentos de terapia avanzada de fabricación no industrial.*

El Reglamento (CE) n.º 1394/2007 del Parlamento Europeo y del Consejo, de 13 de noviembre de 2007, sobre medicamentos de terapia avanzada y por el que se modifican la Directiva 2001/83/CE y el Reglamento (CE) n.º 726/2004, define como «medicamento de terapia avanzada» a los medicamentos de terapia génica, los medicamentos de terapia celular somática, los productos de ingeniería tisular y los medicamentos combinados de terapia avanzada.

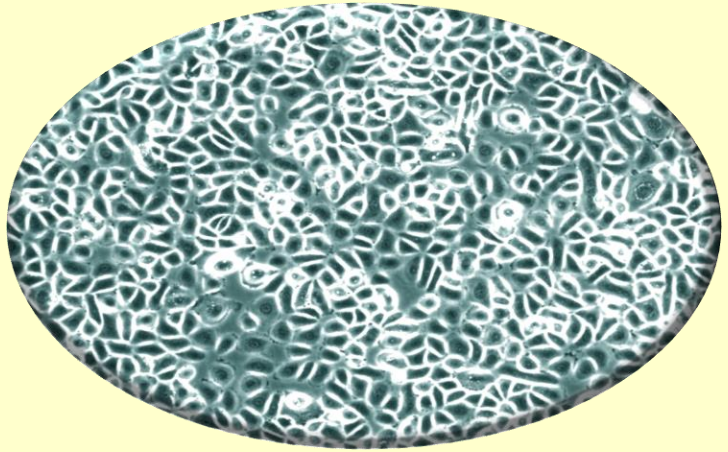
"terapias consolidadas"

condrocitos





queratinocitos



células troncales
limbocorneales

Última actualización: 10/02/2021

El listado que se muestra a continuación contiene las autorizaciones de uso de medicamentos de terapia avanzada concedidas por la Agencia Española de Medicamentos y Productos Sanitarios al amparo del Real Decreto 477/2014, de 13 de junio, por el que se regula la autorización de medicamentos de terapia avanzada de fabricación no industrial.

NOMBRE	TITULAR DE LA AUTORIZACIÓN DE USO	FECHA AUTORIZACIÓN DE USO	FICHA TÉCNICA
NC1- SUSPENSIÓN CELULAR EN PLASMA AUTÓLOGO 100-300×106 CELULAS-HOSPITAL UNIVERSITARIO PUERTA DE HIERRO MAJADAHONDA, 1 jeringa precargada	HOSPITAL UNIVERSITARIO PUERTA DE HIERRO MAJADAHONDA	29-01-2019	FT Ficha técnica pdf
ARI-0001 DISPERSION PARA PERFUSION QUE CONTIENE 0,1-1×10^6 CELULAS/KG – HOSPITAL CLINIC DE BARCELONA	HOSPITAL CLINIC BARCELONA C/Villaroel, 170 Barcelona	01-02-2021	FT Ficha técnica pdf

www.aemps.gob.es/medicamentos-de-uso-humano/terapias-avanzadas/autorizaciones-de-uso-de-medicamentos-de-terapia-avanzada/?lang=en

La sustancia activa de este medicamento son las células mesenquimales troncales adultas autólogas de médula ósea expandidas, a una concentración de 100.000 células/µl.

Excipientes: Plasma sanguíneo autólogo

NC1

3. FORMA FARMACÉUTICA

Suspensión para inyección.

Restringido exclusivamente para uso hospitalari

4. DATOS CLÍNICOS

4.1 Indicaciones terapéuticas

NC1 está indicado en el tratamiento de paciente
medular traumática crónica, que presenten lesio
lumbar.

2.2. Composición cualitativa y cuantitativa

ARI-0001 es una suspensión para infusión que contiene 0,1 -1 x 10^6 células/kg del paciente. Las células se criopreservan en función del peso del paciente. El volumen final es de 30 mL (15 mL de células resuspendidas en suero fisiológico + 15 mL de solución de congelación dimethyl sulfoxide (DMSO al 10% y albúmina al 10% respecto el volumen final).

Excipiente con efecto conocido

Este medicamento contiene 1,78 mg de sodio por ml y 53,3 mg de sodio por dosis.

Para consultar la lista completa de excipientes, ver sección 6.1

3. FORMA FARMACÉUTICA

ARI-0001

Dispersión para perfusión

Restringido exclusivamente para uso hospitalario

4. DATOS CLÍNICOS

4.1. Indicaciones Terapéuticas

ARI-0001 está indicado en el tratamiento de la leucemia linfoblástica aguda (LLA) de células B CD19+ en recaída o refractaria tras un mínimo de dos líneas de tratamiento o en recaída post-trasplante en pacientes adultos mayores de 25 años.

medicamentos de terapia avanzada

autorización de comercialización

investigación clínica

autorización de uso

TerCel | isciii
Red de Terapia Celular

RED TERCEL | NOTICIAS | AGENDA | ENSAYOS CLÍNICOS | SALAS DE PRODUCCIÓN | PACIENTES | CONTACTO

Reunión Técnica TerCel 2021

INGENIERIA CELULAR EN CÉLULAS MESENQUIMALES (MSC 2.0)

TODAS LAS PONENCIAS EN NUESTRA GALERÍA VIRTUAL

¡DISFRÚTALAS AQUÍ!

2021

Buenas Prácticas Clínicas

DESCARGA AQUÍ

EL LIBRO BLANCO DE LA TERAPIA CELULAR EN ESPAÑA

NOTICIAS DESTACADAS

Pluripotencia para la regeneración de órganos

La generación de células madre pluripotentes (hPSC), derivadas de organoides es uno de los mayores logros científicos en medicina regenerativa.. ... *(ver más)*

PRÓXIMOS EVENTOS

www.red-tercel.com

ENSAYOS CLÍNICOS

122

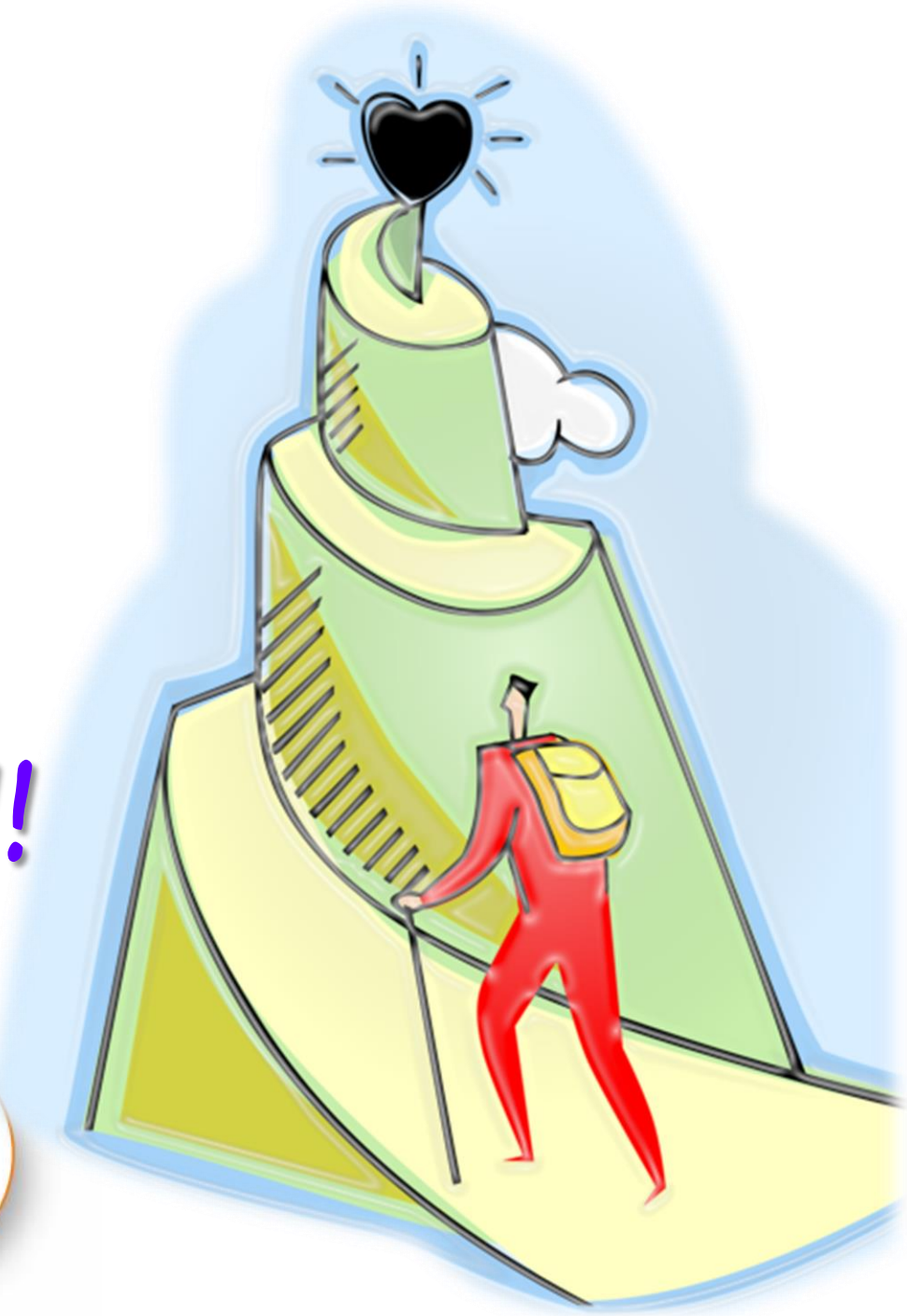
EECC TERCEL

Los Ensayos Clínicos son un pilar esencial en el desarrollo de nuevas terapias basadas en el uso de la Terapia Celular. La Red TerCel ha puesto en marcha numerosos estudios para comprobar la seguridad y la eficacia del uso de células madre en distintas enfermedades. Pinchando en el logo de ClínicalTrials.gov puedes ver los ensayos clínicos registrados en esta plataforma.

What happens once a medicine is marketed

step 06

regulators advice can make a difference!!!



Initial research

step 01

Regulators advice can make a difference!

- **Product classification**
- **Certification of ATMP**
- **SME**
- **Orphan indication**
- **Eligible for PRIME**
- **…**

Human regulatory

Adaptive pathways
Advanced therapies
Clinical trials
Compassionate use
Compliance
Data on medicines (ISO IDMP standards)
Ethical use of animals

PRIME: priority medicines

Share

PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

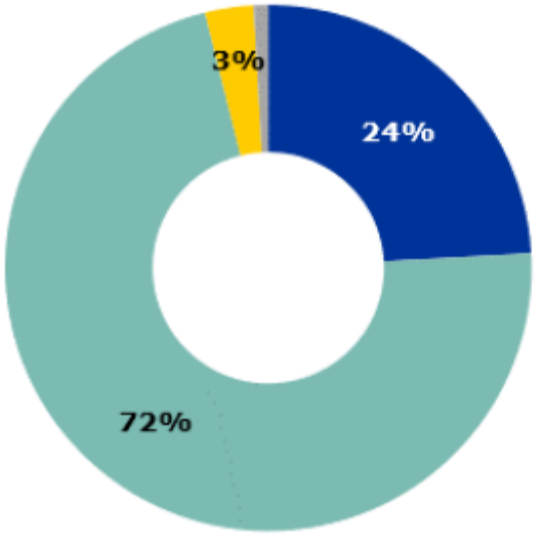
Through PRIME, the Agency offers early and proactive support to medicine developers to optimise the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications.

This will help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

Cumulative overview of PRIME eligibility recommendations adopted by 25 March 2021

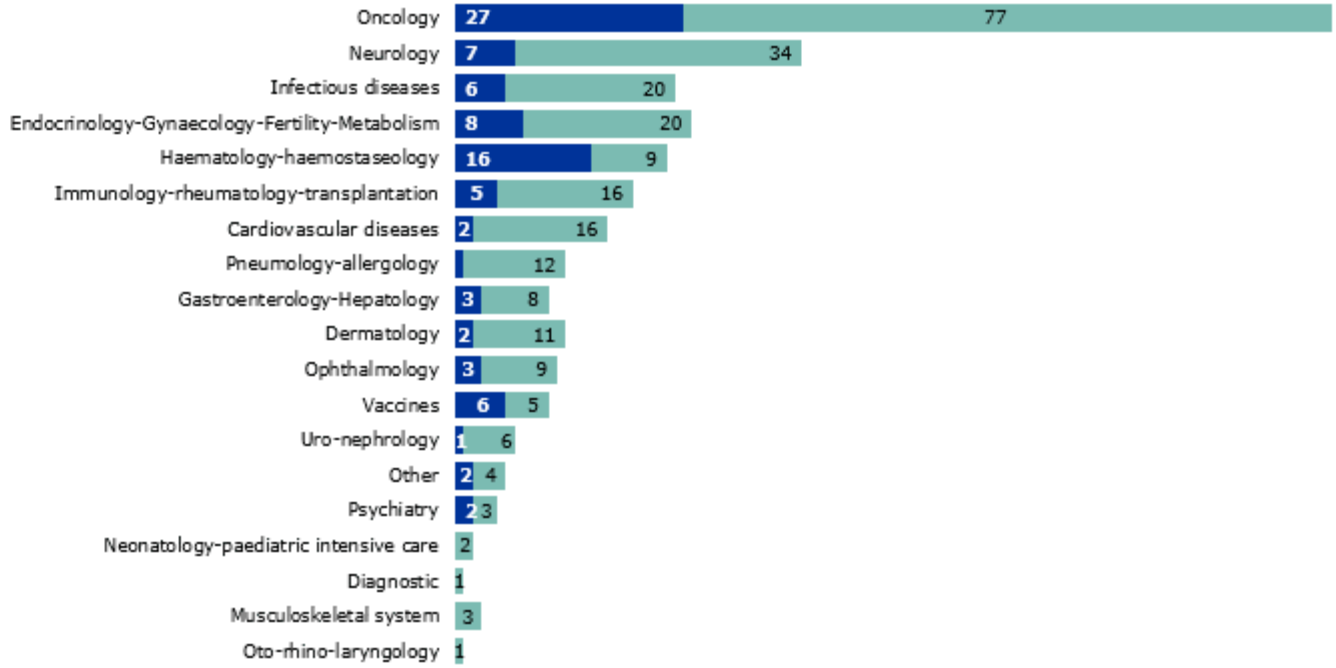
Granted Denied Out of scope*

Overall number



By Applicant Type

By therapeutic area



* This indicates eligibility requests received but not started by EMA as they were deemed outside the scope of the scheme or with a format and content inadequate to support their review. These are not included in the breakdown by type of applicant or by therapeutic area.

www.ema.europa.eu/en/human-regulatory/research-development/prime-priority-medicines

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant	Date of granting PRIME eligibility
Adeno-associated viral vector serotype 8 containing the human MTM1 gene (AT132)	Advanced therapy	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of X-linked Myotubular Myopathy	Nonclinical + Clinical exploratory	SME	31/05/2018
Adenovirus associated viral vector serotype 5 containing the human RPGR gene	Advanced Therapy	Ophthalmology	Treatment of X linked Retinitis Pigmentosa owing to defects in Retinitis Pigmentosa GTPase Regulator	Nonclinical + Clinical exploratory	Other	27/02/2020
Adenovirus associated viral vector serotype 8 containing the human CNGB3 gene (AAV2/8-hGARp-hCNGB3)	*Advanced therapy*	*Ophthalmology*	*Treatment of achromatopsia associated with defects in CNGB3*	*Nonclinical + Tolerability first in man*	*Other*	*22/02/2018*
ADP-A2M4 (transduced CD4+ and CD8+ cells)	Advanced Therapy	Oncology	Treatment of HLA-A*02 positive patients with inoperable or metastatic synovial sarcoma who have received prior chemotherapy and whose tumour expresses the MAGE-A4 tumour antigen	Nonclinical + Clinical exploratory	Other	23/07/2020
Allogeneic EBV-specific Cytotoxic T Lymphocytes	Advanced Therapy	Oncology	Treatment of rituximab refractory Post-Transplant Lymphoproliferative Disorder (PTLD)	Nonclinical + Clinical exploratory	Other	29/05/2019
Allogeneic multi-virus specific T lymphocytes targeting BK Virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus, and adenoviru	Advanced Therapy	Infectious Diseases	Treatment of serious infections with BK virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus, and/or adenovirus in allogeneic HSCT recipients	Nonclinical + Clinical exploratory	Other	30/01/2020
AT-GTX-501 (adeno-associated viral vector, serotype 9, containing th human CLN6 gene)	Advanced Therapy Medicinal Product	Neurology	Slowing disease progression in paediatric patients with variant late infantile neuronal ceroid lipofuscinosis 6 (vLINCL6)	Nonclinical+ Clinical exploratory	Other	17/09/2020
Autologous anti-CD19/CD20 CAR T transduced cells (MB-CART2019.1)	Advanced Therapy	Oncology	Treatment of patients with relapsed and refractory diffuse large B-cell lymphoma (DLBCL) after frontline therapy and who are ineligible for autologous stem cell transplantation	Nonclinical + Clinical exploratory	Other	17/10/2019

Academia developing medicines for rare diseases to receive free EMA scientific advice

Share

News 23/06/2020

To further encourage the development of treatments for rare diseases, EMA will waive all fees for scientific advice for academia developing orphan medicines.

The academic sector plays an important role in the development of innovative medicines. Their scientific research is often at the source of novel methodologies and innovative medicines with a potential to benefit patients with rare diseases.

Early interaction with EU regulators is important for academia to understand the regulatory requirements and allow the generation of robust evidence needed to establish the medicines' benefits and risks. This helps them to navigate the regulatory process and ultimately to translate their discoveries into authorised, patient-focused medicines.

However, feedback from academia showed that fees for protocol assistance (scientific advice for orphan medicines) represent a hurdle to engage with EMA.

In light of these findings and the actions foreseen in the Agency's framework of collaboration with academia, the Regulatory Science Strategy to 2025 and the EMA SME action plan, EMA decided to include academia in the list of organisations eligible for free protocol assistance as of 19 June 2020 .

Compartir:

Última actualización: 28/07/2016

Puede consultar esta nota en formato pdf

OFICINA DE APOYO A LA INNOVACIÓN Y CONOCIMIENTO SOBRE MEDICAMENTOS

Fecha de publicación: 28 de julio de 2016

medicamento de terapia avanzada

DEBE CUMPLIR LA LEGISLACIÓN DE MEDICAMENTOS

- **autorización:** comercialización, investigación clínica, uso compasivo…
- garantías de **calidad, seguridad, eficacia**
- son aplicables **GMP** (producción y control), **GLP** (estudios no clínicos) y **GCP** (ensayos clínicos)

aemps

- investigación clínica, uso compasivo
- autorización de uso (*cláusula de exclusión*)

ema

autorización de comercialización en la UE

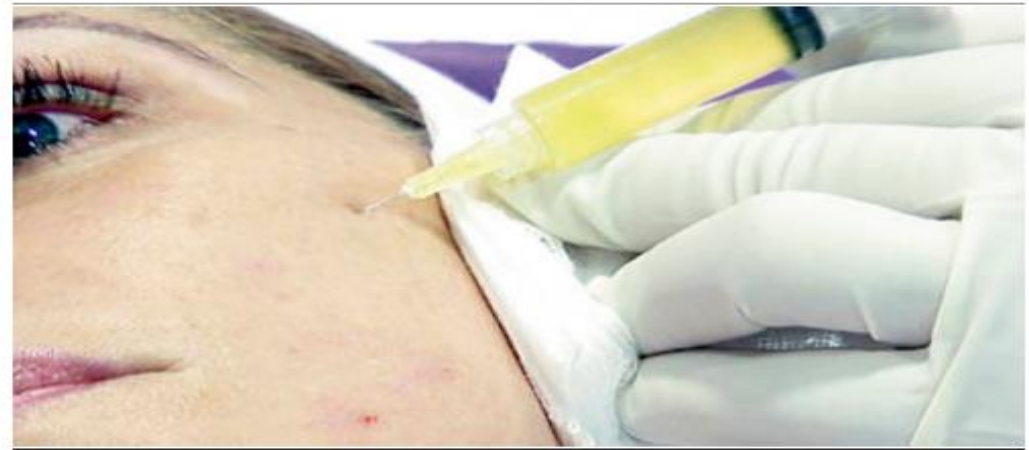
LA RAZÓN.es

MEDICINA Y SANIDAD

Células madre para borrar las arrugas

- El tratamiento con células madre masenquimales, responsables de producir colágeno, elastina y ácido hialurónico, está indicado para el rejuvenecimiento facial. A diferencia de otros métodos de relleno, no produce rechazo

Like 0
Twittear 0
Pin it
Share



Células madre para borrar las arrugas

14 de octubre de 2012. 08:00h

B.M. - Madrid.

El empleo de células madre ha supuesto un antes y un después en la Medicina. A su uso en la regeneración de tejidos u oncología se suma ahora la estética .

MINISTERIO DE SANIDAD Y POLÍTICA SOCIAL

agencia española de medicamentos y productos sanitarios

Nota informativa

Advertencia sobre la oferta de tratamientos no autorizados basados en el uso de células madre

16 de abril de 2010

La Agencia Española de Medicamentos y Productos Sanitarios (AEMPS) tiene conocimiento de la oferta directa a ciudadanos y pacientes de diferentes tratamientos basados en la manipulación de células madre y postulados para un amplio espectro de enfermedades, en general enfermedades graves y crónicas. Todas las agencias nacionales europeas comparten la preocupación por este hecho y ello ha llevado a que la Agencia Europea del Medicamento (EMA) haga pública una nota *(Concerns over unregulated medicinal products containing stem cells*, EMA/763463/2009) en la que se alerta de la oferta a pacientes de medicamentos con células madre no regulados.

La AEMPS quiere trasladar e
siguientes consideraciones pa
tales prácticas:

MINISTERIO DE SANIDAD, SERVICIOS SOCIALES E IGUALDAD

agencia española de medicamentos y productos sanitarios

Nota informativa

Agencia Española de Medicamentos y Productos Sanitarios
AEMPS

LA AGENCIA ESPAÑOLA DE MEDICAMENTOS Y PRODUCTOS SANITARIOS ADVIERTE SOBRE LA POSIBLE CONFUSIÓN EN LA OFERTA DE TRATAMIENTOS CON CÉLULAS MADRE

Fecha de publicación: 22 de octubre de 2012

Categoría: AEMPS, MEDICAMENTOS DE USO HUMANO, MEDICAMENTOS ILEGALES, COSMÉTICOS.
Referencia: AEMPS, 10/2012

La Agencia Española de Medicamentos y Productos Sanitarios quiere advertir sobre la posible confusión en la que se pueda caer ante la utilización profusa de términos relacionados con las terapias basadas en células madre para situaciones tan dispares como el tratamiento de enfermedades o el uso de cosméticos.

MINISTERIO DE SANIDAD, CONSUMO Y BIENESTAR SOCIAL

agencia española de medicamentos y productos sanitarios

Nota informativa

Agencia Española de Medicamentos y Productos Sanitarios
AEMPS

CONSIDERACIONES SOBRE LOS PRODUCTOS SANITARIOS UTILIZADOS PARA LA OBTENCIÓN DE CÉLULAS AUTÓLOGAS Y LA CLASIFICACIÓN DEL PRODUCTO RESULTANTE COMO MEDICAMENTO DE TERAPIA AVANZADA

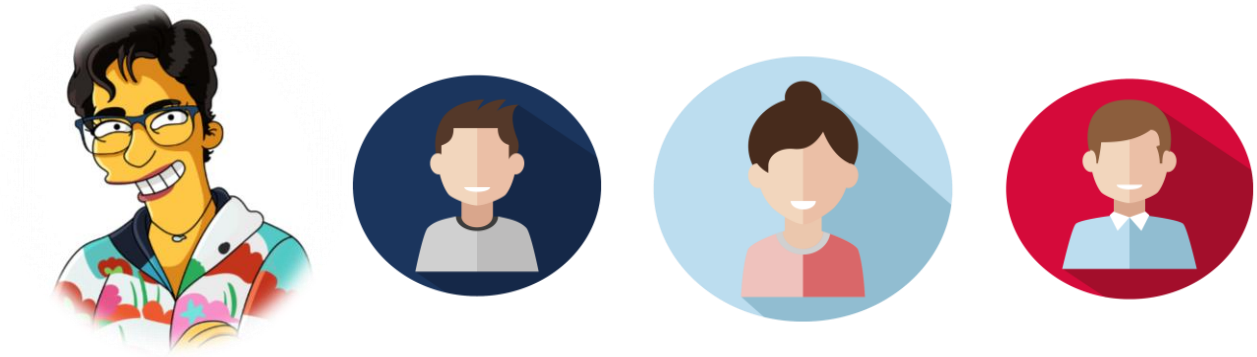
Fecha de publicación: 12 de julio de 2018

Categoría: MEDICAMENTOS DE USO HUMANO, PRODUCTOS SANITARIOS
Referencia: MUH, 4 /2018

La Agencia Española de Medicamentos y Productos Sanitarios informa sobre la consideración que tienen los productos sanitarios que se ofrecen para obtener células autólogas, así como el producto final obtenido.

www.aemps.gob.es/medicamentos-de-uso-humano/terapias-avanzadas/

¡Muchas gracias!



sruiz@aemps.es