



Rare Diseases 360
Collaborative Strategies to leave no-one behind

9th European Conference on Rare Diseases & Orphan Products
10-12 May 2018 Vienna

Global Rare Equity: are we there yet?

Rare Diseases impose common problems and strong equity challenges to Health Systems and social protection, in the mark of distributive justice. Latin America does not escape from these challenges, which intensify due to our demographical, geographical, economic, cultural, and social characteristics.⁽¹⁾





Rare Diseases 360°
Collaborative Strategies to leave no-one behind

9th European Conference on Rare Diseases & Orphan Products

10-12 May 2018 Vienna

Global Rare Equity: **Argentina** are we there yet?

All my speech is about to built collaboratives strategies and the strengths and opportunities we have to offer to materialise them.

The strengths of the two national ministries

The Health Ministry

The Science Ministry

To close I present a case of a collaborative strategy to access to innovative therapies

Finally what we lack



Key enblers:

- Argentina has 55 internationally recognized National Universities that receive students from all Latin America.
- Argentina is a country with an open economy, receptive to international dialogue and accesible to the development of advanced therapies. Argentina has an innovative Science and Technology Ministry linked to the most important scientific institutions of the world.
- Argentina has consolidated network of advocacy patients, validated by theirs protagonist and recognized of its representativeness.

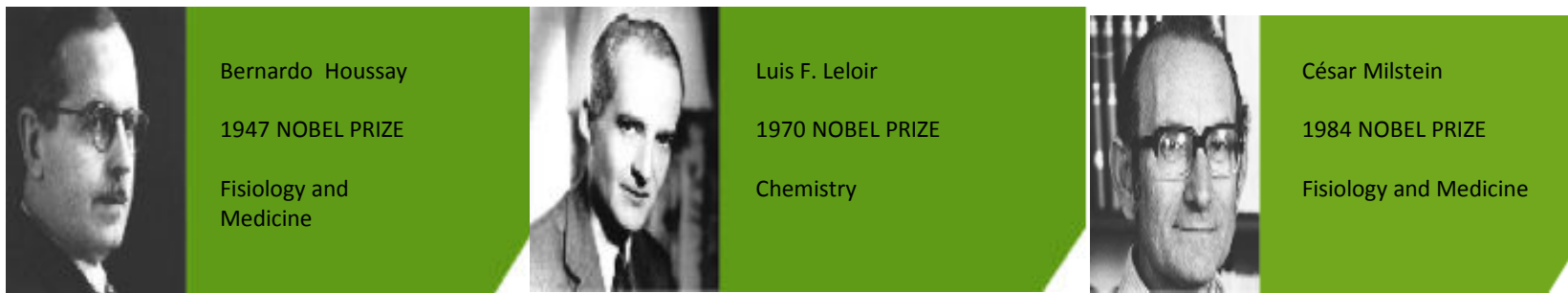


Key enablers:

A long tradition of excellence in scientific development allowed the formation of a highly qualified human resources

Three Argentinean scientists awarded with Nobel Prize

Argentina is a country with a prestigious career in medical sciences that has 3 Nobel prizes and we are looking for 4th !!!





Opportunities in our health sphere:

- National Law 26,689 of Rare Diseases
- National Program of RD
- National Registry of RD
- Access to therapies
- RedApta





Opportunities in our health sphere:

➤ National Health Ministry

ENFERMEDADES POCO FRECUENTES
Y ANOMALÍAS CONGÉNITAS

Ministerio de Salud
Presidencia de la Nación

Comunicador: 54-11-4379-8000
Av. 9 de Julio 1925 (C1073ABA)

INICIO INSTITUCIONAL INFORMACIÓN PARA CIUDADANOS EQUIPOS DE SALUD RECURSOS DE COMUNICACIÓN CONTACTOS

24 octubre, 2017
La importancia del ácido fólico antes del embarazo
El consumo de ácido fólico antes de un embarazo ayuda a prevenir los defectos del cierre del tubo neural, Anencefalia y Mielomeningocele (Espina Bifida). En nuestro país se produce 1 caso cada 2.000 nacimientos.
[Leer Más ...](#)

Buscar ...

CURSO INTERACTIVO
sobre Enfermedades Poco Frecuentes
[+ INFORMACIÓN](#)

Centro Nacional de Genética Médica
[+ INFORMACIÓN](#)

[+] Banco de recursos EQUIPOS DE SALUD

Mapa de Recursos EPF y Anomalías Congénitas
Servicios de Genética en la Argentina
Mapa de Recursos EPF y Anomalías Congénitas

Anomalías Congénitas
Enfoque para la Atención Primaria de la Salud
Anomalías Congénitas
Atención Primaria de la salud.

REPORTE ANUAL 2016
Reporte Anual Renac 2016
Recién Nacidos.



Opportunities in our health sphere

Time lines
Public health policies

2007	2009	2011	2015	Current challenges
Creation of the Genetics Program	RENAC: National Registry of Congenital Anomalies	National Law of Rare Diseases: 26,689 (18 Jurisdictions adhered)	Regulation of the National Law <ul style="list-style-type: none"> ➤ Creation and implementation of the National RD Program, Ministerial Resolution 2339/14 ➤ Genetic Program ➤ RD area ➤ Treatment Area of Growth Hormon 	<ul style="list-style-type: none"> ➤ National Registry RD (SIISA) ➤ Consultive advice cometee ➤ List of RD



Opportunities in our
health sphere:



Website of the Ministry of Health
Orphanet Argentina



Annual RD Courses by Telehealth
RD in Primary Care in person



Management leaders by province
Identification of RD expertised centres



Advice and guidance to families, health teams (through mail, telephone and face-to-face calls)
Joint work with NGOs
In-person orientation and counseling interviews with the National Director of NGOs



Preparation of reports
Responses to requirements
Growth hormone Program
Training, material for the website



Opportunities in our Science sphere:

- Argentina understood the importance to accelerate the safe development of regenerative medicine



- Today implements GLOBAL REGULATORY AFFAIRS



Opportunities in our Science sphere:

➤ Legal & regulatory framework in Argentina

Transplants Act

- Ley 24.193

Regulatory
Ministry of Health

- Resolución 610-2007
 - Res. 307/2007
 - Dcto 512/95
- Resolución N° 119/2012

Authority:
INCUCAI

***Professional Practice
Medicine Act 1967***

Medicines Act

- Ley 16.463

Regulatory
Ministry of Health
/ANMAT

- Disposición 7075/2011
- Disposición 7729/2011

Authority:
Ministry of Health/ANMAT

Patient Rights Act 2009

Clinical trials

Regulatory
Resolución 6677/2011
(Clinical Research)

Authority:
Ministry of Health/ANMAT



Opportunities in our Science sphere:

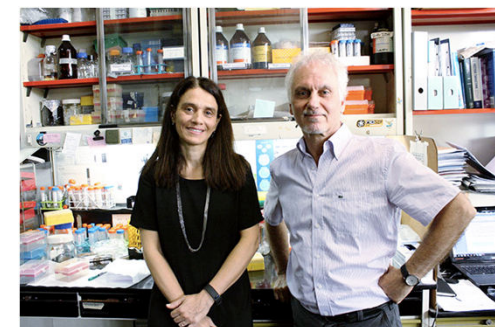
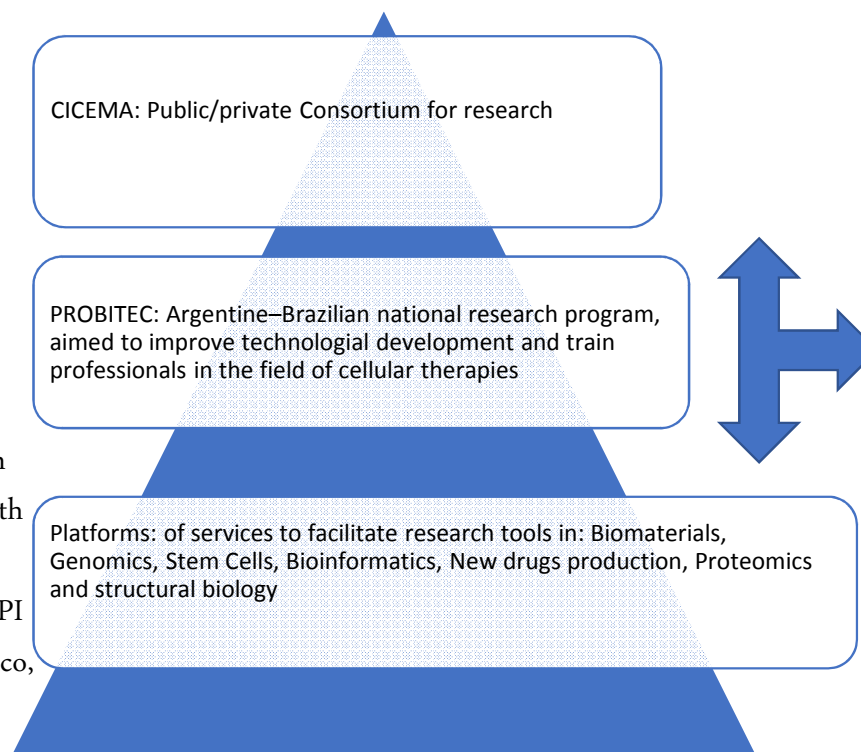
- Ministry of Science, Technology and Productive Innovation
- Advisory Commission Regenerative Medicine and Cellular Therapies (2006)
- Minister: Lino Barañao; Chair: Fabiana Arzuaga
- Peralta, Jorge; Pitossi, Fernando; Podhajcer, Osvaldo; Saidón, Patricia; Seoane, Martín; Sevliver, Gustavo; Sommer, Susana; Bergel, Salvador; Coco, Roberto; Del Pozo, Ana Luna, Florencia; Kusminsky, Gustavo; Miriuka, Santiago





Opportunities in our Science sphere:

- RED APTA
- Creation of a new Coalition of patients
- This network is called RED APTA (Argentinean Patients Network for Advanced Therapies). With International collaborations: University of Edinburgh, McGill University, CIRM (USA), GPI (USA), France, Belgium, Colombia, Chile, México, Perú, Germany, etc.





Opportunities in our Science sphere:

Open debate with all stakeholders

- Releasing of joint announcements with scientific/professional medical societies (Diabetes, Neurology, Paediatrics, Ophthalmology, etc) with a clear message about stem cell treatments state of the art.
- Interactions with journalists to help them understand which is the real status of cell therapies to communicate with more objectivity.
- Interactions with Ministry of Health

Argentina create the conditions to promote Regenerative Medicine in our country and provide a regulatory framework to research advanced therapies that facilitates the generation of safe and effective treatments for patients in harmony with international markets.





A case in progress:

what's next?

PAMPA



- Consortium-Coalition for Precision Medicine with official support and endorsement (*Ministries of Health and Science Tech and Innovation*) framed in the National Precision Medicine Programme


 Consejo Nacional de Investigaciones Científicas y Técnicas


 INSTITUTO LOLOIR
FUNDACIÓN




 UNIVERSIDAD AUSTRAL


 Ministerio de Salud
Presidencia de la Nación


 Ministerio de Ciencia, Tecnología e Innovación Productiva
Presidencia de la Nación


 U.S. EMBASSY
ARGENTINA





Strengthening Health Systems Through Sustainable Interventions

Increase regional newborn screening practices: evaluate the population of newborns screening practices for rare disorders. These actions to be carried out to increase the equity and to improve the practice of the neonatal screening process for rare diseases in the region.

Education and Awareness: There is a poor conscience about the presence of RD and conditions of the communities suffering these diseases and absence of education in the health sciences universities. Add to the topic in School of Medicine and the health care professionals. Invite medical societies to participate

Harmonised data & digital technology: The lack records becomes impossible to divulge information about these sufferings, to know the pathology, and, through innovation, how to handle the phenomenon with treatments adequate to the patients. (DHIs) Classification of Digital Health Interventions v 1.0





GLOBAL EQUITY

- ❖ **Interdisciplinary**
- ❖ **Collaborative team work**
- ❖ **Health networks**
- ❖ **Concerted Alliances**





THANKS A LOT!!!!!!

clarisa_marchetti@hotmail.com

Rare Diseases 360°
Collaborative Strategies to leave no-one behind

9th European Conference on Rare Diseases & Orphan Products

10-12 May 2018 Vienna





Rare Diseases 360°
Collaborative Strategies to leave no-one behind

9th European Conference on Rare Diseases & Orphan Products
10-12 May 2018 Vienna

Sources & sponsor

- Romina Armendo MD. *National Ministry of Health*
- Lino Barañao Ph. *National Ministry of Science & Tecnology*
- Fabiana Arzuaga Ph. *REDAPTA*
- Podhajcer, Osvaldo Ph. *CONICET*
- Florencia Braga Memendez; Juan Capria MD. *Research Fundation*
- Elicier Quispe. *MPS Ecuador*
- [\(1\) http://press.ispor.org/LatinAmerica/2017/09/rare-diseases-in-latin-america-challenges-and-opportunities-for-the-equitable-attention-and-proposal-of-patients-organizations/](http://press.ispor.org/LatinAmerica/2017/09/rare-diseases-in-latin-america-challenges-and-opportunities-for-the-equitable-attention-and-proposal-of-patients-organizations/)