

Rare Diseases in Argentina

(and in most of Latin America)

A view from the medical and scientific field

Emilio J. A. Roldan, MD, PhD

Qualix DoT, Scientific Director
CERyDH, General coordinator
ICORD, board member

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About Argentina

- Population 44,5 M (Rare Diseases 2,2M?)
- Annual inflation around 40%; GDP -10,2; unemployment rate 11.7% (economic and social crisis) 2019/2020 data
- Education: high literacy level (80% pop); 1,5M students at University;
- High Sci and Tech in medicine, biotech, nanotech
- Health Care System: 7,5% GDP; 37,4% pop has no health insurance; 48,8% covered by around 300 social services (union), 8,6% has private insurance plans (200 organizations)

A problem of positioning...

Which is the place of RDs at the public health and science agenda?

Law 26.689 (100% coverage for RDs)

No National program

No specific fundings

Is “low prevalence” a negative definition?

Detour the problem. The core is the rareness not the quantities of affected people.

Put off political priority and interest.

A problem of scientific/industrial logistic

- Lack of RDs agenda in the research system
- Lack of orphan products in the R&D industrial agenda (only generics or copies).
- Lack of regulatory guiding or facilitation for RDs R&D. (harmonization with the region, tax exceptions, bonus, etc.)
- Difficulties in trial recruitments (isolation, diagnoses, managements)

A problem of scientists encouragement

- There is no incentives for the scientist (You get only what is incentivized)

Scientific carrer based on publications scores and citation indexes. Hence massive topics are preferred.

- No traslation to industry (below 1% of science expenditures are translated; very low patent rate)
- Scientific and industrial environments are mostly diconnected.

A problem of health care managements.

- RDs experts works isolated and barely associated to networks (with exception for specific conditions).
- Health care resources are good but not adequately distributed.
- Awareness is poor within the primary health care professionals.
- Awareness is poor for prevention of RDs.
- Genetic services are improving but still not enough.
- Accessibility to orphan drugs and diagnoses is low.

Argentina and LA&C (Latin America and Caribbean)

- Argentina is geographically, cultural and historically inserted within the LA&C countries. The complexity of the RDs demands a regional vision.
- Regional network of health care experts and researchers
- Coordination of regional scientific programs
- Regulatory harmonization for RDs

The conclusion at present...

- Argentina (and LA&C) needs to be inserted within the international RDs cooperation initiatives.
- Argentina needs to learn from the experiences of countries more advanced in the field.
- Argentina needs a National RDs program (cost/efficiency optimized)
- Argentina needs to coordinate its science services with its health care in order to increase its translational rate, and include RDs.
- Argentina needs incentives to track its sci & tech resources to the cooperation within the international community.

The conclusion at present...

- There is much to be done...
- Lets start quickly...cooperating.

What about the patient's activities?

- Mrs Ana Maria Forte

Journalist. Her radio program “Hablemos de Salud” en great part devoted to rare diseases.

Founder of Familiar Metiranean Fever, Argentina

What about the patient's activities?

Como me involucre con las ERs?

How did I get involved with RDs?

Necesidades de pacientes con ERS

Needs of patients with RDs.

- Relación medico pacientes
- Mayor tiempo de consultas
- Preparación para la detección precoz
- Centro de referencias

- Relationship doctor and patients.
- Longer consultation time.
- Preparation for early diagnoses.
- Reference Centers.

What about the patient's activities?

Necesidades de pacientes con ERS

- Tratamientos consensuados.
- Apoyo integral para familiares.
- Ayuda para las organizaciones.
- Ayuda para el trabajo voluntariado.
- Mayor apoyo desde la industria.

Needs of patients with RDs.

- Consensual treatments.
- Integral support for caregivers.
- Support for patients' groups.
- Support for volunteers.

- Greater support from the industry.