

VI ICORD

Session XII

International initiatives on rare
diseases and orphan drugs

ARGENTINA

Emilio J.A. Roldán

SLADIMER - ARGENTINA

Where do we stand on (2010)?:

- LEGISLATION
- EDUCATION
- ASSISTANCE
- AWARENESS
- BASIC RESEARCH
- CLINICAL RESEARCH
- REGULATOR
- INDUSTRY

- LEGISLATION
- A National Law now at the Parliament
- Provincial laws projects at the City of Buenos Aires (3.111, Nov 2009),
- projects in Entre Rios and Rio Negro.

- EDUCATION
- No official programs at any University (Public or private)
- GENETIC not recognized as an independent university chair
- RD issues included in few postgraduate meetings.

- ASSISTANCE

- No RD reference centre (one recently created at CABA)
- No local network
- Lack of info on international networks
- Social security, lack of specific regulations
- Lack of family programs
- Lack of social rehabilitation to QOL services/programs

- **AWARENESS**
- CPA mass media campaign project
- Good relationship with press
- Government of CABA established the “RD week”
- Agreement with academia to encourage RDs education.

- BASIC RESEARCH
- Well prepared researchers, but...
- No master plan
- No specific funding
- No links – ISOLATION
- Gov/academia negotiations, with few intervention of industry and almost none of patient organizations.

- CLINICAL RESEARCH
- Only foreign industrial trials on-going
- Poor knowledge of small sample size trials management
- Bioethics – vulnerability aspects poorly considered
- No specific regulations for orphan drugs

- REGULATORY
- No Dept. of Orphan drugs
- No definition of Rare Diseases
- No public list of approved trials available
- GCP, audits in progress
- Pharmacovigilance rules in progress

- LOCAL INDUSTRY
- Good resources, but...
- Poor knowledge of the market features
- Not clear channels of distributions and reimbursement policy
- No incentives
- No master plans (poor connection with academia and governments)
- Looking mainly at the national markets (but starting to change)

My (subjective) scoring of current situation (from 0 to 10)

- GOVERNMENT 2
- PATIENT ORGANIZATIONS 4
- ACADEMIA 4
- INDUSTRY 2

CONCLUSION:

we have a lot of work to do.