

# Centres of Expertise

## The Portuguese situation



EURORDIS membership meeting - Madrid - 2015/05/30

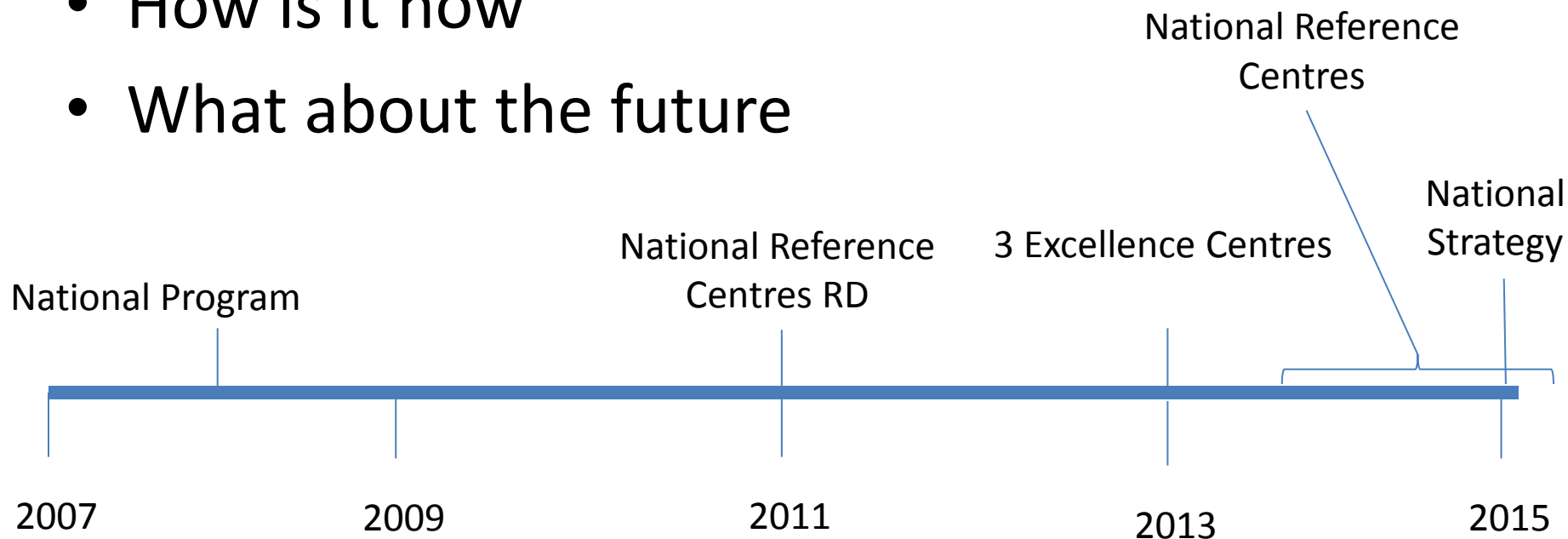
?

**BAD IS NEVER  
GOOD UNTIL  
WORSE HAPPENS.**

Danish proverb

# Agenda

- How was it
- How is it now
- What about the future



# The past – 2008

## **National Program for Rare Diseases approved**

(<http://www.portaldasaude.pt/NR/rdonlyres/555DD3B3-45F0-4F74-B633-28889E721BF1/0/i010420.pdf>)

- Public discussion and meetings with
  - Rare disease patients
  - Healthcare professionals
  - Researchers
  - Industry

# The past – 2008

## National Program for Rare Diseases

- Pros
  - Concrete proposals
  - Great coverage of RD issues (including Reference Centres)
  - Issued before recommendation 2009/C 151/02
- Cons
  - Only part of it was further studied in groups
  - Only one of the groups had patient representatives
  - Almost none of it was implemented
  - No budget defined

# The past - 2011

## National network of Reference Centres for Rare Diseases approved

(<http://www.portaldasaude.pt/portal/conteudos/a+saude+em+portugal/politica+da+saude/discussao/arquivo/doencas+raras.htm>)

- Pros
  - Related to the Nacional Program
  - Within the time frame
- Cons
  - Never implemented

# The past - 2013

## **Creation of 3 excellence centres (despacho 2545/2013 - dispatch)**

[http://www.insa.pt/sites/INSA/Portugues/AreasCientificas/Genetica/Documents/Anexo\\_1\\_despacho\\_2545\\_2013.pdf](http://www.insa.pt/sites/INSA/Portugues/AreasCientificas/Genetica/Documents/Anexo_1_despacho_2545_2013.pdf)

- **Puzzling!**
  - Only for lysosomal diseases
  - “excellence” instead of “reference”
  - Why these 3?
  - Not related to the national network approved in 2011
- **Status unknown**

# The present - 2014

## **Regulation on the healthcare National Reference Centers (Portaria 194/2014 - ordinance)**

([http://www.sanchoeassociados.com/DireitoMedicina/Omlegissum/legislacao2014/Setembro/Port\\_194\\_2014.pdf](http://www.sanchoeassociados.com/DireitoMedicina/Omlegissum/legislacao2014/Setembro/Port_194_2014.pdf))

- Overview
  - RD diagnose and treatment included
  - Definition, how to identify them
  - Procedure for approval and recognition
- RD specificity is lost



# The present - 2014

## **National Commission for the Reference Centres elected (despacho 13163-C/2014 - dispatch)**

<http://www.spdm.org.pt/wp-content/uploads/2013/11/Despacho-n.%C2%BA-13163-C-2014.pdf>

- People from several entities (governmental)
- No patient representatives

# The present - 2015

**Definition of the priority intervention areas for which Reference Centres must be recognized in 2015 (despacho 235-A/2015 - dispatch)**

[http://www.sanchoeassociados.com/DireitoMedicina/Omlegissum/legislacao2015/Janeiro/Desp\\_235A\\_2015.pdf](http://www.sanchoeassociados.com/DireitoMedicina/Omlegissum/legislacao2015/Janeiro/Desp_235A_2015.pdf)

- 6 groups of diseases
- Only 2 “rare diseases” on the RD group

# The present - 2015

- Cancers in adults:
  - Cancro do esófago
  - Cancro do testículo
  - Sarcomas do adulto
  - Cancro do reto
  - Cancro hepato-bilio/pancreático
- Cancers in pediatric patients:
  - Doenças Hemato-Oncológicas
  - Hepatoblastomas
  - Tumores do Sistema Nervoso
  - Onco-Oftalmologia
  - **Neuroblastoma**
  - Sarcomas oftálmicos
  - **Wilms tumor**
  - Tumores ósseos
- **Refractory Epilepsy**
- Cardiovascular diseases:
  - Cardiologia de “intervenção estrutural”
  - Cardiopatias congénitas
- Transplantations:
  - Fígado
  - Rim
  - Coração
  - Pulmão
  - Pâncreas
- **Rare Diseases:**
  - **Metabolic hereditary diseases**
  - **Familial Paramyloidosis**

# The present - 2015

## **Integrated Strategy for Rare Diseases 2015-2020** (despacho 2129-B/2015 - dispatch)

([http://www.inr.pt/uploads/docs/noticias/2015/despachdo\\_estrategia\\_doencas\\_raras.pdf](http://www.inr.pt/uploads/docs/noticias/2015/despachdo_estrategia_doencas_raras.pdf))

- Revokes
  - National Program
  - National network of Reference Centres for RD

# The present - 2015

## **Integrated Strategy for Rare Diseases 2015-2020** (despacho 2129-B/2015 - dispatch)

- Pros
  - The timeframe of the National Program was almost over
- Cons
  - No public consultation
  - No previous work with the various stakeholders (Aliança was given the document during a meeting for 30min)
  - No concrete proposals/measures
  - No financing model for the strategy implementation

# The present - 2015

## **Integrated Strategy for Rare Diseases 2015-2020** (despacho 2129-B/2015 - dispatch)

- ?
  - Health, Social Security and Science and Education Ministries together
    - Urbanism among others can be included
    - Some that might be imported are not to be included (some health and education institutions)
  - DGHealth is the head of a inter ministerial body – on what grounds?
  - No executive body... can the commission enforce the measures to be defined?

# The future

# The future

- Uncertain
- EUROPLAN conclusions to be announced
- Orphanet PT
- Rare disease patient card
  
- Reference centres...



# Thank you!



Doenças Raras Portugal

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