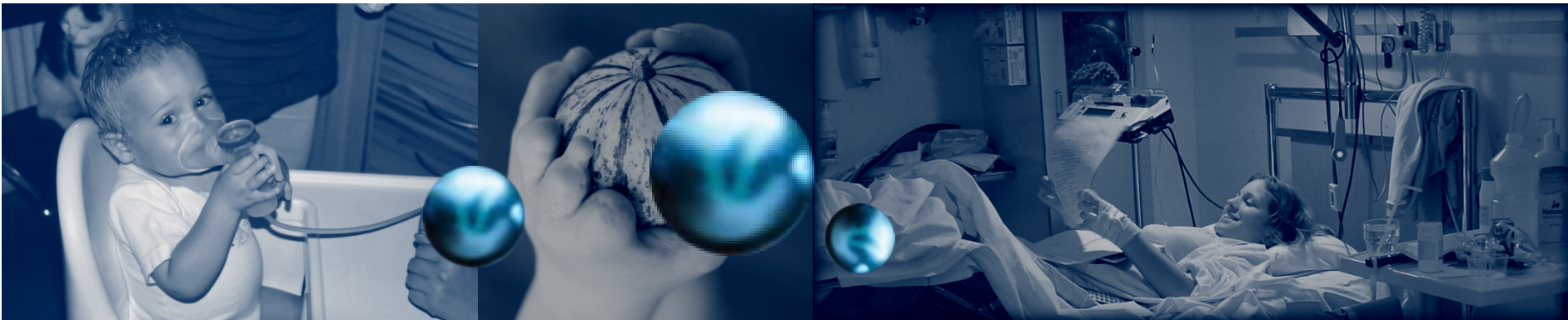


# Recruitment of patients in rare diseases trials worldwide



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Director of Treatment Information and Access

25 April 2013



# Background

- The EMA is collecting important information on the recruitment of CT subjects (all diseases)
  - In the EU / EEA / EFTA, there was a decrease from 37.0%\* in 2005 to 31.2% in 2011 (**huge impact HTA**)
  - Within this region, the contribution of the 15 countries that were members of the EU before May 2004 plus Norway, Iceland and Liechtenstein fell from 32.1% to 19.4% over the observation period
  - The contribution of the countries that became Member States of the EU in 2004 and 2007 increased from 4.7% in 2005 to 11.7% in 2011.

\*: % of subjects recruited in the EU/EEA/EFTA countries



# Background

- The DITA task force reviews the EMA report every time it is published
- In 2009, the DITA asked the EMA to perform the same analysis with CTs for orphan drugs
- This data was entered in 2009
- And is therefore available for all trials submitted 2010 onwards



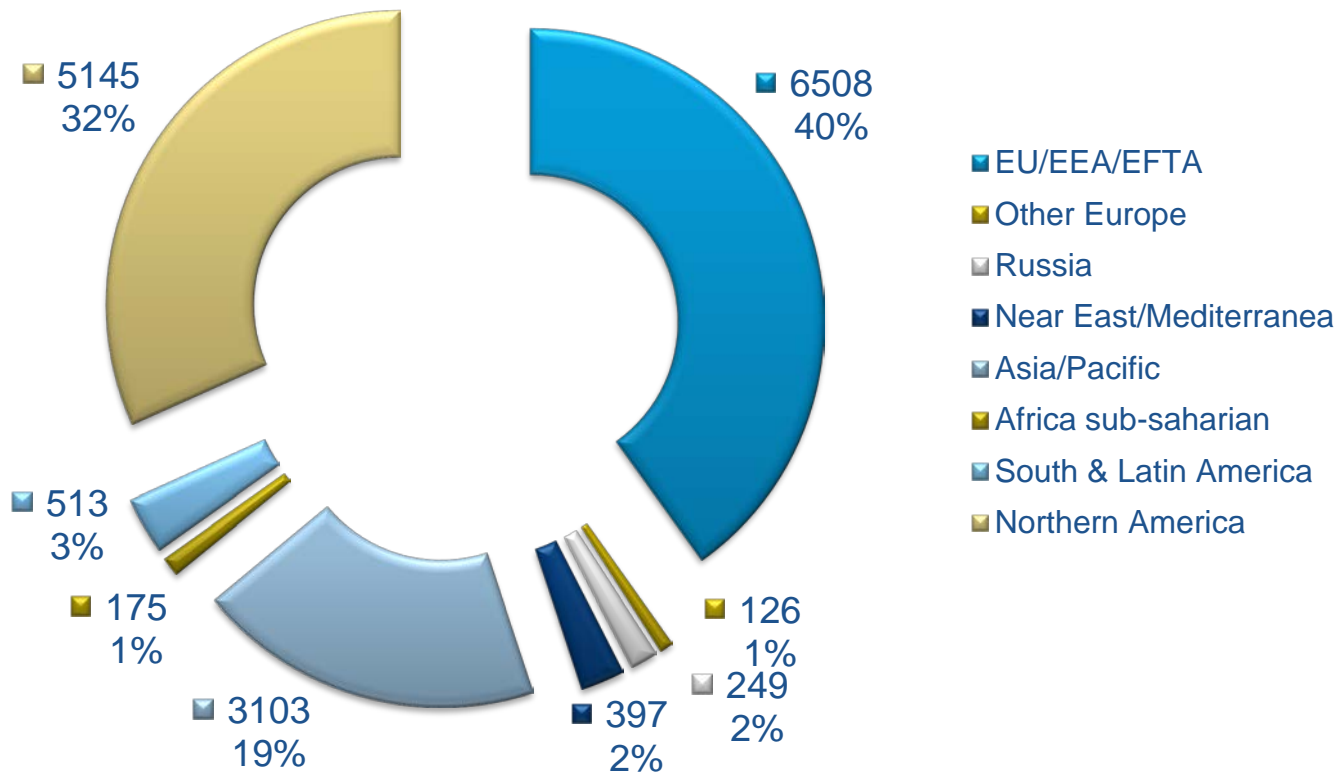
# Source of data

- **Thank you to:**
  - Ana Rodriguez Sanchez & Miguel Rodriguez
  - Clinical and Non-Clinical Compliance and Inspection section, EMA
  
- **Period**
  - In 2010 and 2011, the EMA received 225 applications with CT reviewed by the section
  - out of them 42 were for Orphan products
  - Of the 54 pivotal clinical trials used in these 42 applications, only 13 pivotal studies did not have any patients in the EU/EEA/EFTA area.



# Total subjects in RD: 16216

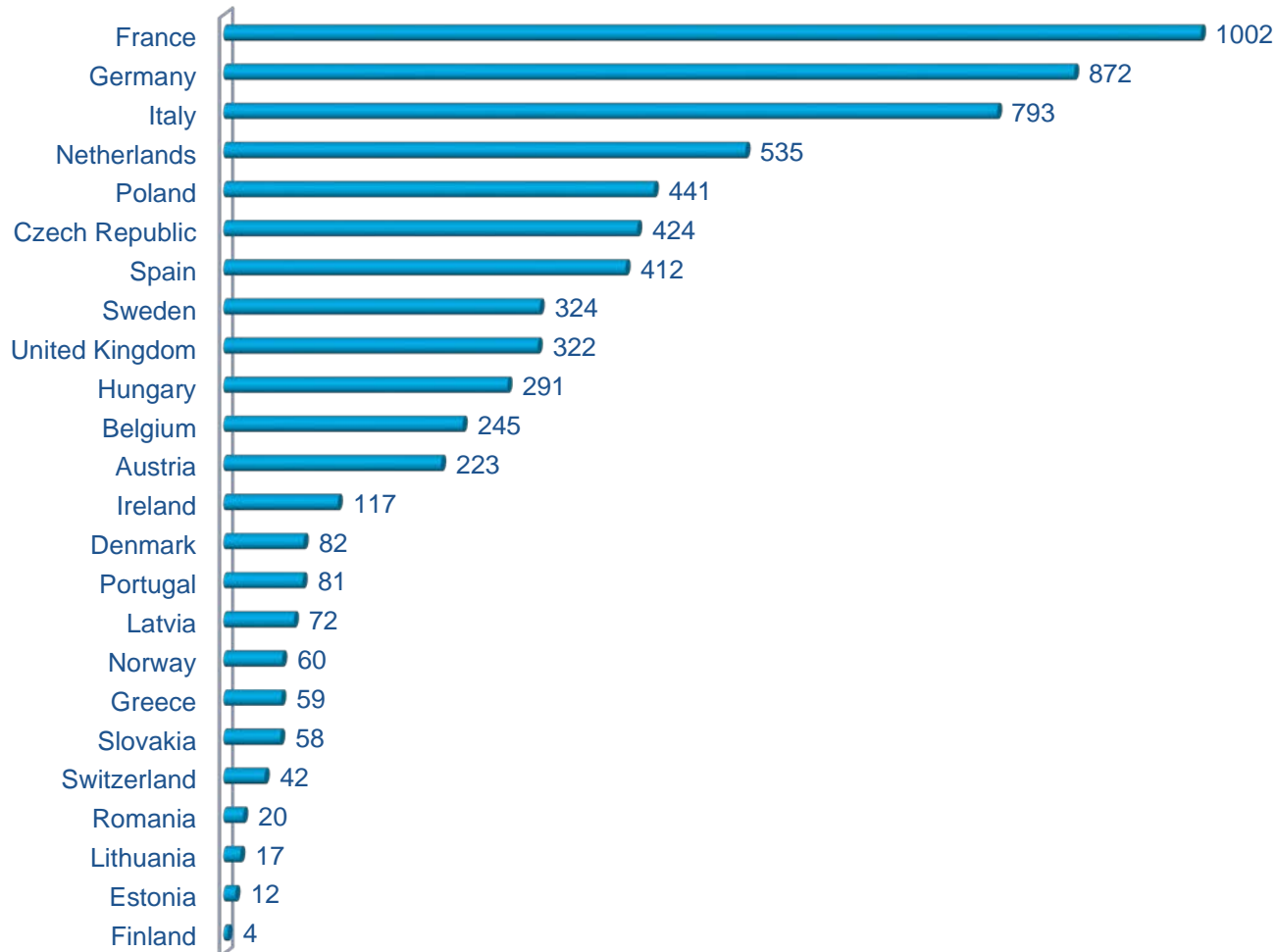
## By world regions





# Number of subjects in EU / EEA / EFTA countries

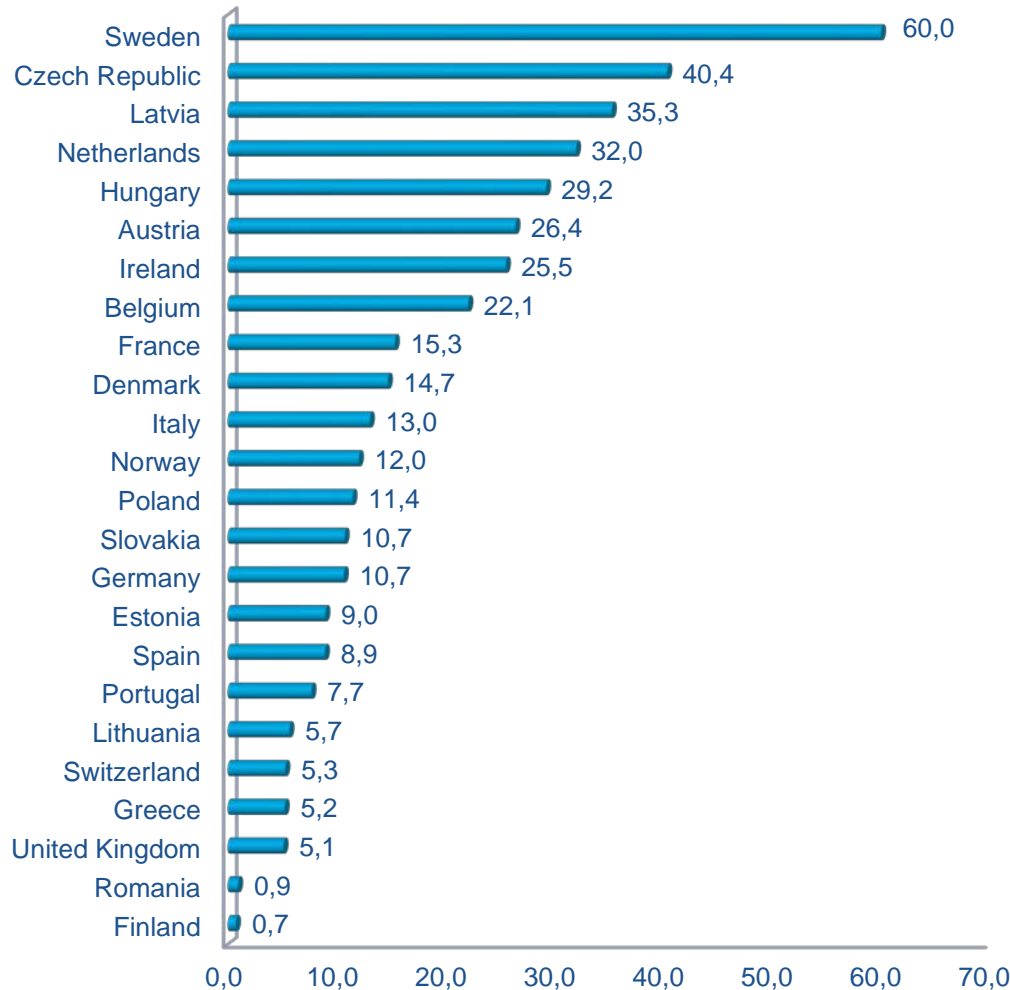
## EU/EEA/EFTA





# Number of subjects in EU / EEA / EFTA countries

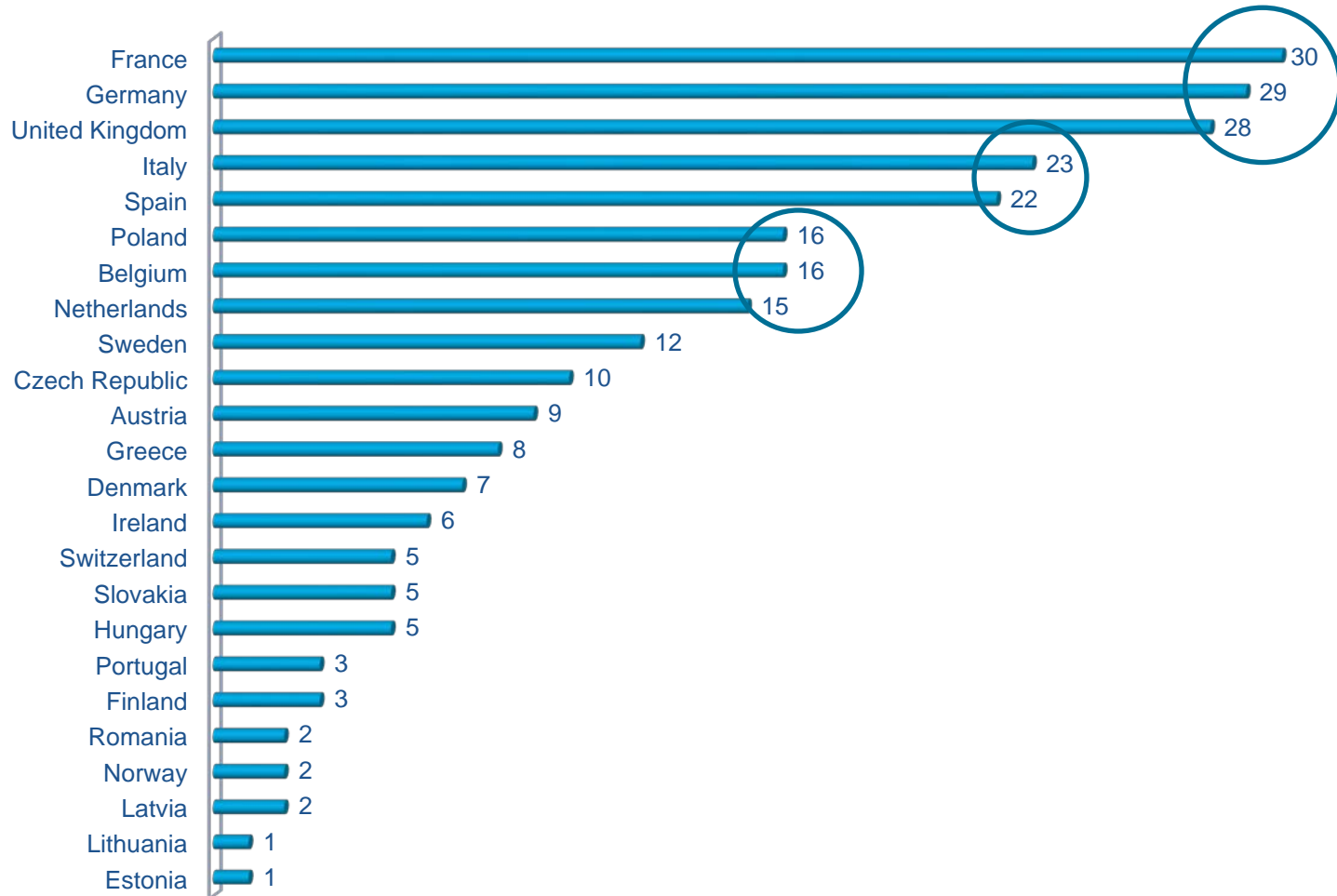
per million pop





# Number of CTs in EU / EEA / EFTA countries (41 trials in this region)

## EU/EEA/EFTA

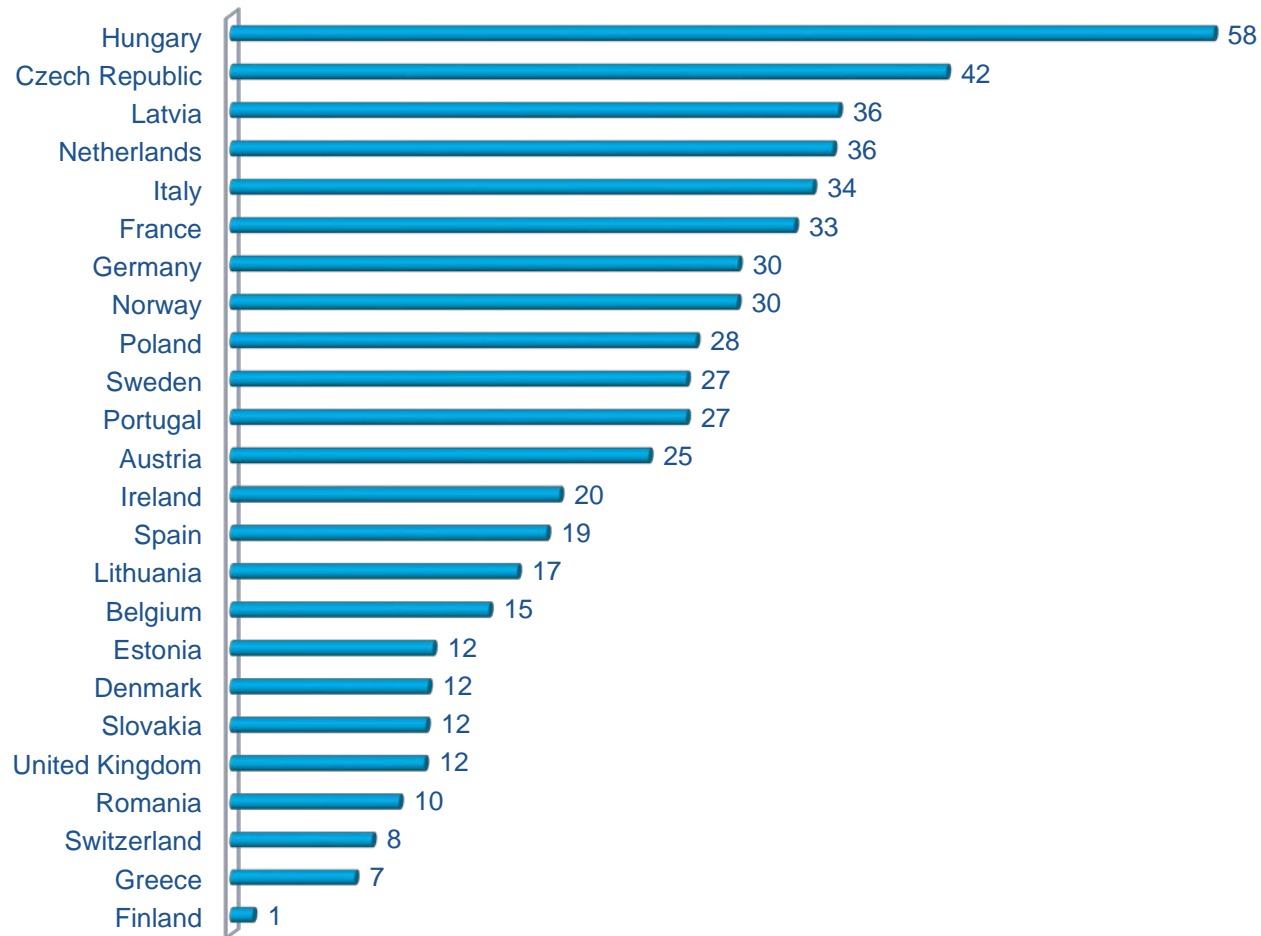






# Numbers of patients by trial

## Patients/CT

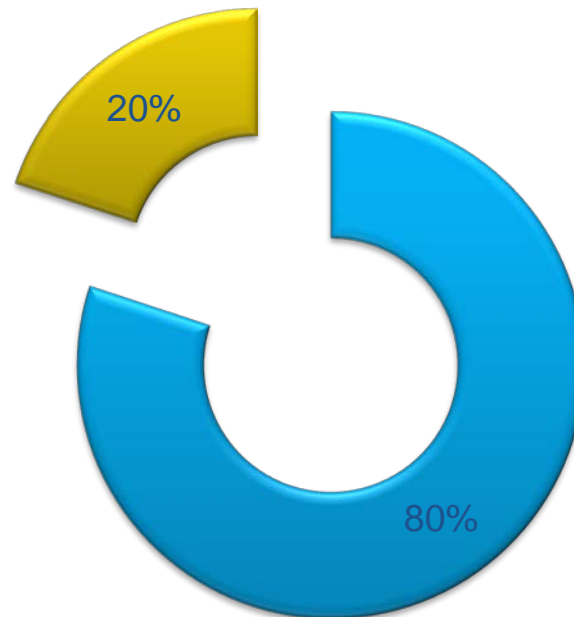




# Distribution in the EU

## Within EU

■ EU 15 (ante 2004) ■ new MS

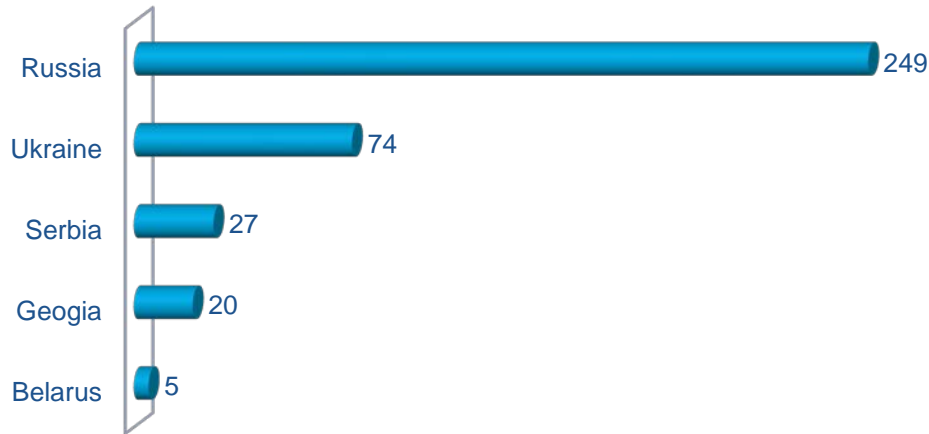


For all CTs, the distribution is 65% (EU 15) versus 35%.

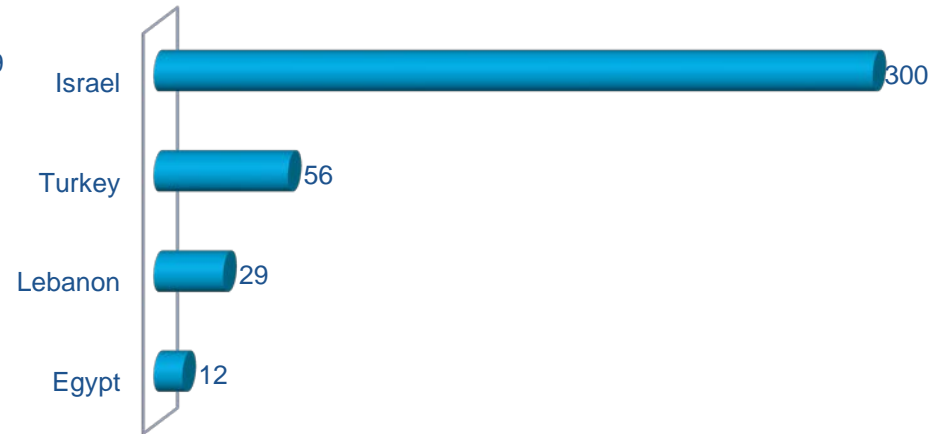


# Number of subjects in RofW

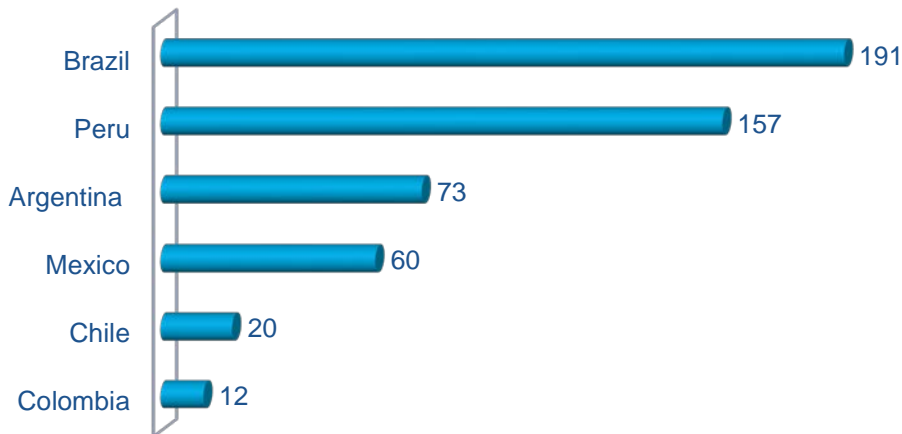
## Other Europe



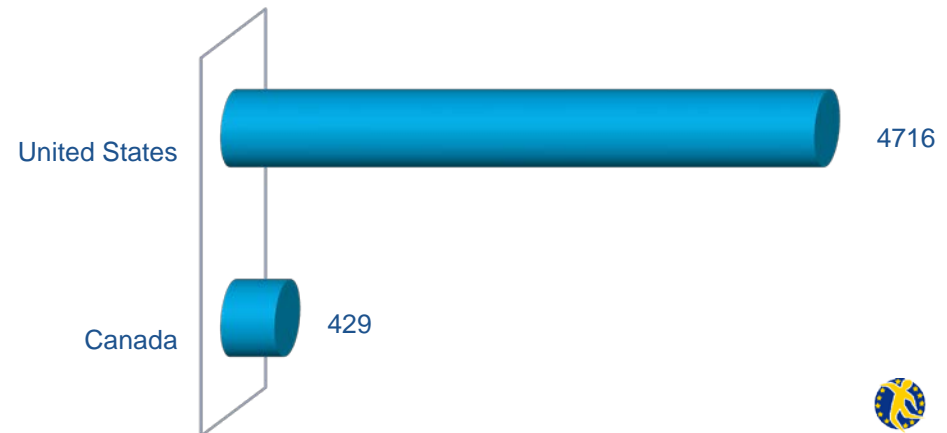
## Near East/Mediterranea



## South & Latin America



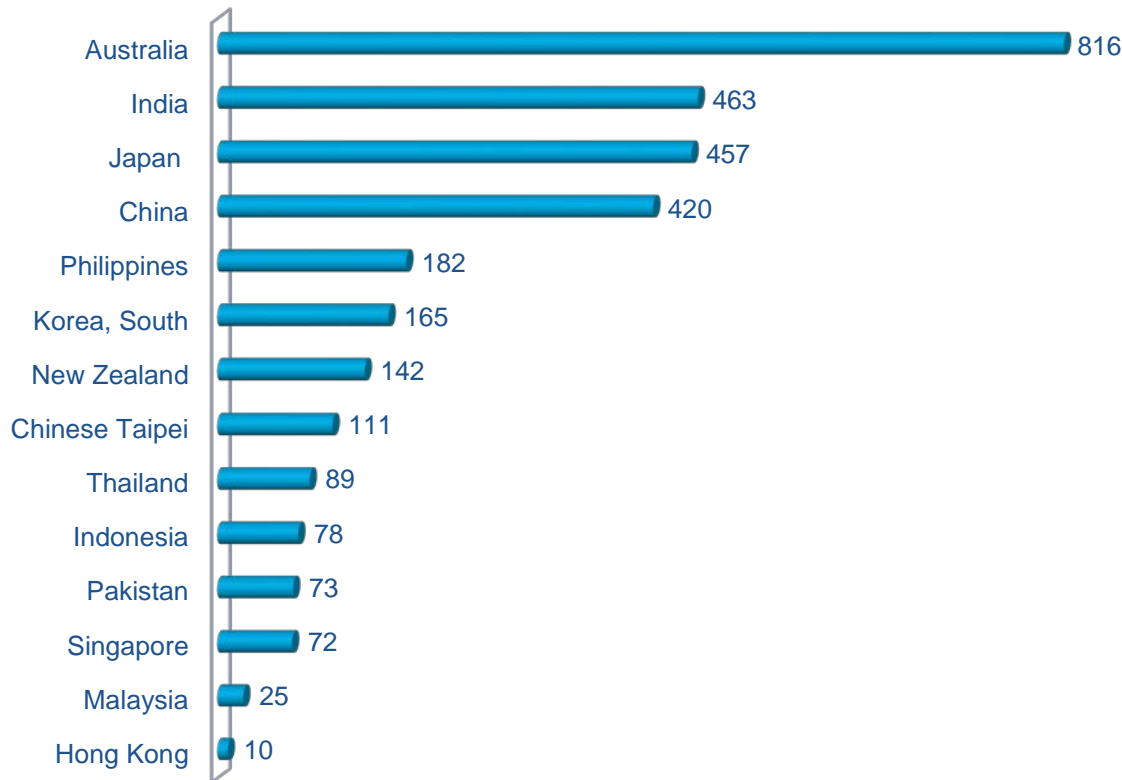
## Northern America





# Number of subjects in RofW

## Asia/Pacific



Africa sub-saharian	
South Africa	175



# Comments

- The % of patients in CT for orphan drugs applications are more or less in line with the historical % for all pivotal CT submitted to the Agency.
- For countries outside EU/EEA/EFTA, USA, Canada, Japan and Australia
  - These group represents 13,051 subjects (80.48%)
  - The other countries with a significant number of subjects are India, China and Israel.
- Please note that numbers are very low and in a small period of time, a large CT will make the % to change considerably.