EARLY ACCESS TO MEDICINES IN EUROPE: COMPASSIONATE USE TO BECOME A REALITY

Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP)

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A frequent situation

New drug being developed

New drug authorised

Marketing authorisation

Some patients have no more treatment options, their condition deteriorates. Some die. They know a product is being developed.

When the drug is authorised, patients can have access.

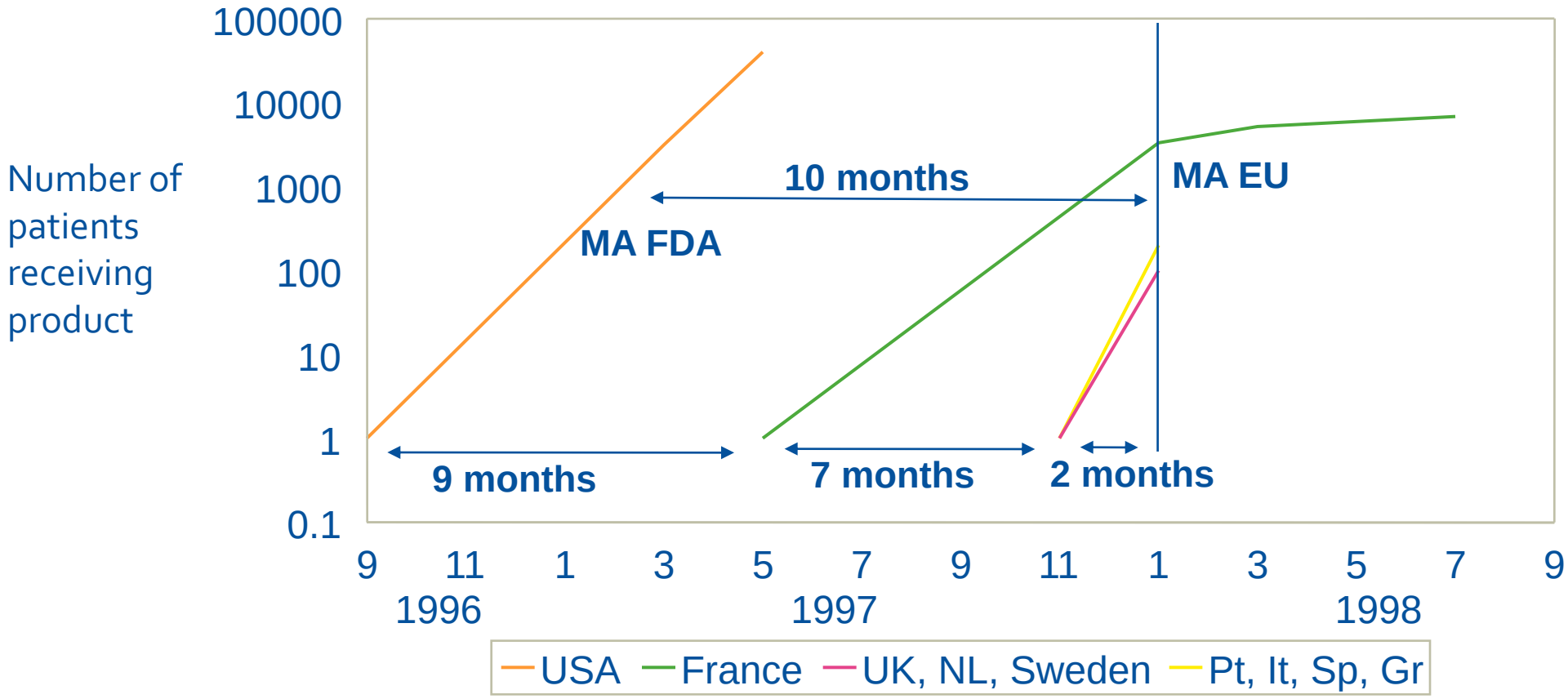
There is always one patient who will suffer the day before a drug is authorised and who knows the drug will be authorised next day

For all, this is a nightmare

REGULATION (EC) Nº 726/2004 art. 83

- *Defines what compassionate use programmes are*
- *Explains the conditions (the medicinal product concerned must either be the subject of an application for a marketing authorisation or must be undergoing clinical trials)*
- *Organises the CHMP opinion on MS request*
- *Requests MS to notify to EMA CUP they authorise*
- *Requests CUPs to be continued during the period between authorisation and placing on the market*

Historically, access to compassionate use highly heterogeneous in the EU (e.g. nelfinavir in 1997, as shown to CHMP)



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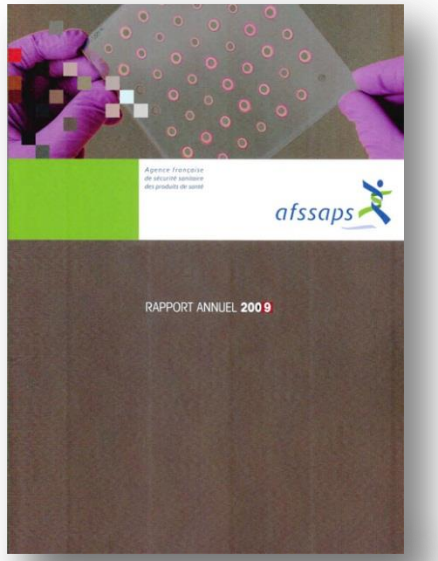
A.T.U* and orphan drugs

- Afssaps (now ANSM), annual report 2009

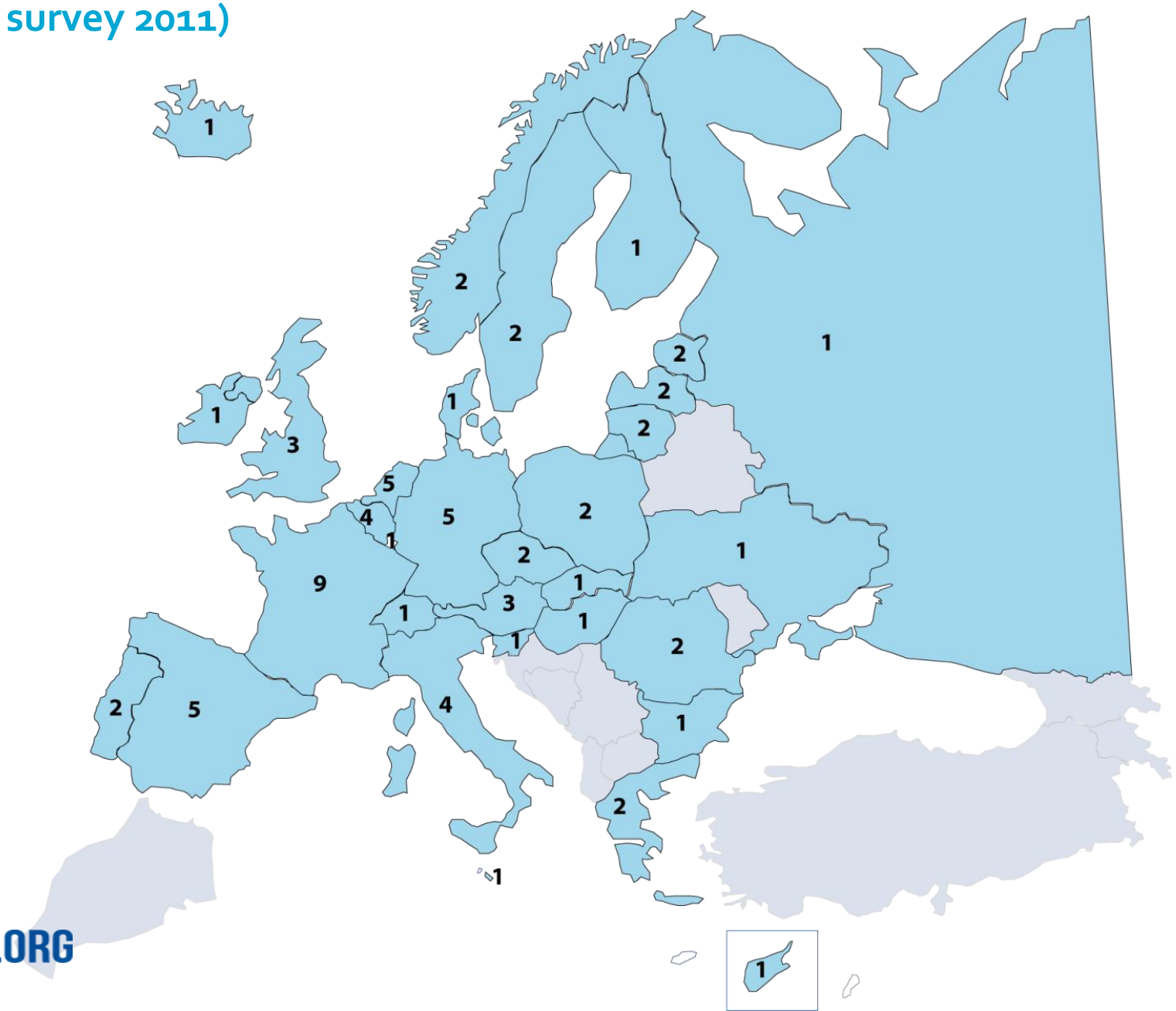


- 72% of authorised orphan drugs received ATU* status
- In average 34 months before their authorisation via the centralised procedure

* Temporary Use Authorisation

9 products, 42 countries, 74 programmes

(Eurordis survey 2011)



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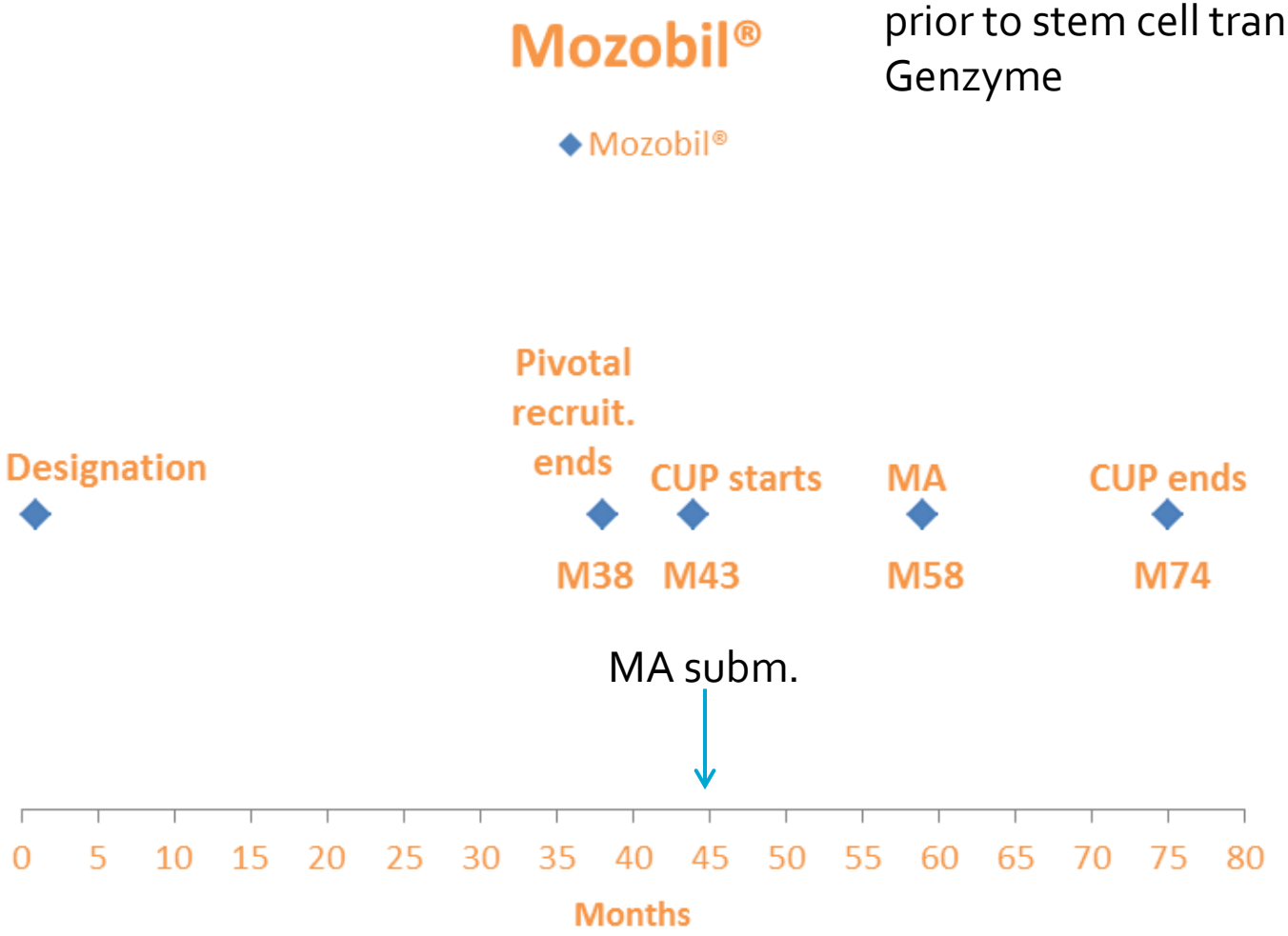
RARE DISEASES EUROPE

Completed programmes (1)
(Eurordis survey 2011)



prior to stem cell transplantation
Genzyme

7

Completed programmes (2)
(Eurordis survey 2011)

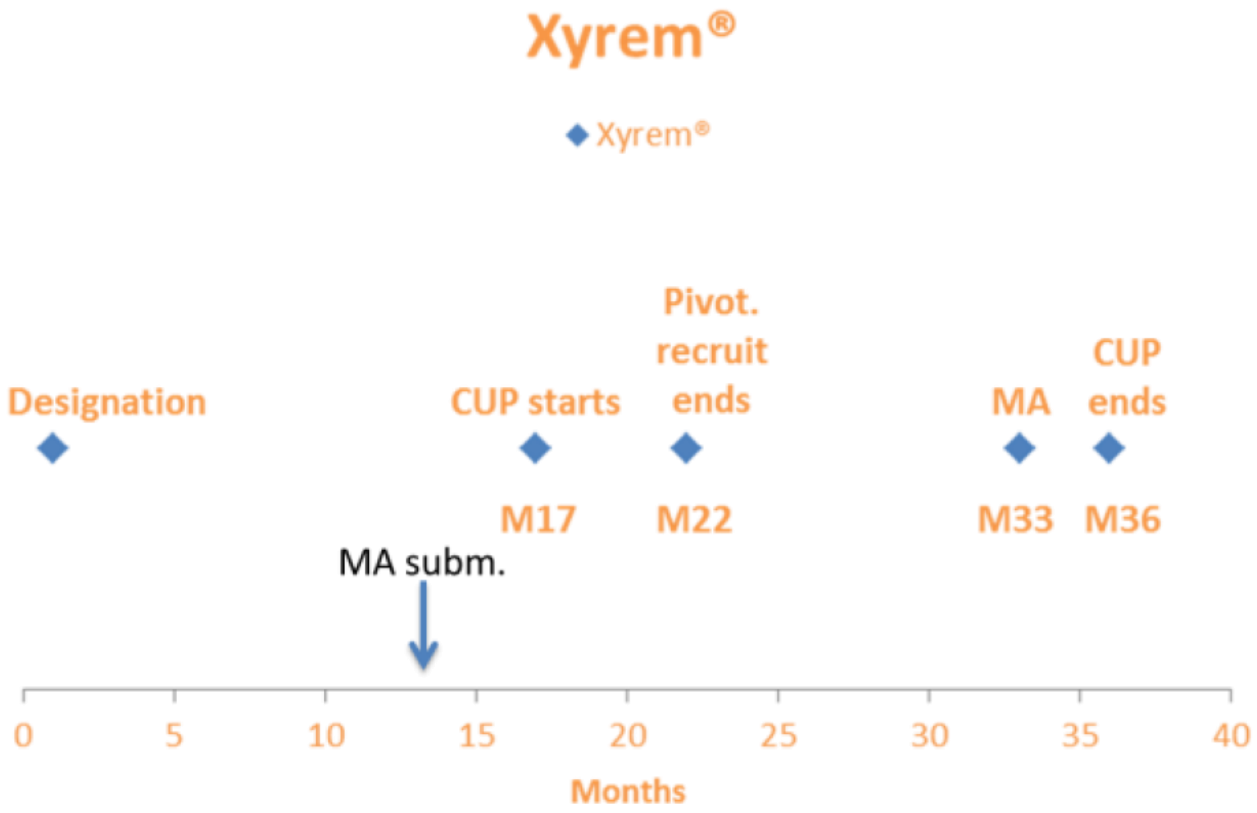


Figure 1: CUP for Xyrem® to treat narcolepsy

Challenges with the current situation

EU legislation

- Objective of the legislation
  - Harmonisation
  - Common approach between MSs
  - Equal treatment for patients across the EU

Not met

- Role of the EMA (*may* provision)
  - Evaluation
  - Opinion (*may* adopt an opinion)
- Role of the MSs final decision (take account)

Main concerns

- Differences between MSs policies difficult to understand (authorisation, documentation, prescription, assessment time, validity, follow up)
- Interference with the marketing authorisation procedure and whether or not the data collected in a programme can be part of the dossier submitted to regulatory authorities
- Liability risks
- Lack of transparency
- Supply and logistics, information/language
- Pressure on supply under compassionate use including off-label
- Free of charge/prices

Hell is in the details

Germany

- CUP to be initiated by the company, not by clinicians
- CUP must be for free
- CUP must use commercial batches of highest quality
- Unclear who pays for other expenses (surgery…)

France

- CUP can be on doctor's request
- CUP can be free of charge or paid for
- CUP can use pilot batches
- Healthcare system pays for all related expenses

Another hot topic: when supply is limited

- Equity:
  - Members of patients' organisations should not be advantaged compared to non-members (no advantage for the best informed)
  - Medical criteria: doesn't work, doctors write what they want
  - If extremely limited supply: random draw to enrol patients

- French Ethics Council Opinions 1996

  Since patients will be selected randomly by computer, there will be no conscious or unconscious emotional preference or pressure. Drawing lots will relieve doctors of the responsibility of choice and preserve patients' trust in their attending physicians. Lots will be drawn each time supplementary drug doses are made available, with the aim of including all eligible patients.

What EURORDIS is proposing

Policy options (not mutually exclusive)

- Promote the French ATU system
- Adopt an EU Regulation / amend article 83
- Apply the Directive on Patients' Rights in Cross-Border Healthcare
- Generalise the Medicines Adaptive Pathways to Patients
- Amend the EMA guidelines for compassionate use

Revise EMA guidelines on CUP, as proposed in ComCom on RD in 2008

- EMA guidelines: very restrictive interpretation of the Regulation
  - CHMP adopt opinions on the conditions for use, the conditions for distribution and the patients targeted
- EMA interprets conditions for distribution only as
  - Medicinal product is subject to medical prescription, or whether it is subject to special or restricted medical prescription.
- What should be addressed by EMA
  - Anticipation of the programme during early SA
  - Estimates on how many patients could benefit from the CUP in the EU
  - Criteria to increase the number of patients when more product is available
  - Measures when the demand exceeds available supply
  - Measures to ensure a fair distribution of available stock among Member States

In addition to policy proposals, 28 recommendations to:

- Patients' organisations (3)
- Industry (18)
- Member States (4)
- European Authorities (EC, EMA, HMA) (3)

Conclusions

- A facilitation group should be set up with MSs
- To improve transparency: MS to respect Regulation (EC) N° 726/2004 and notify the EMA
- To involve patients as it's done in most of the areas dealing with medicines
- MSs have rules in place that can be improved and harmonised via guidelines to be set up in common
- In order to give access to new medicines for those that need particularly for orphan drugs

Thank you for your attention.

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