EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Key concepts of orphan designation and latest developments

SME Workshop 26 April 2013

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Head of orphan medicines

Outline

Overview orphan designation

Orphan designation procedure and criteria

Incentives for orphan drugs

Practical considerations for SMEs

A “dream” example

Latest developments

Purpose of the Regulation

- To provide incentives for research, marketing and placing on the market designated orphan medicinal products
- To set up system for designation of orphan medicines

Main characteristics orphan designation

For medicinal products for human use

Procedure free of charge

Can be requested at any stage of development

Sponsor can be either company or individual

- Established in the EEA (EU, Ice, Liech, Nor)

European Commission Decision gives access to incentives

Incentives (I)

- Fee reduction / exemption (annually reviewed)
  - Extended incentives for Small and Medium Sized Enterprises (SMEs)
- Market exclusivity (10 years)
- Protocol assistance
- Community marketing authorisation
- National incentives (inventory from European Commission)

Incentives (II)

10-year market exclusivity

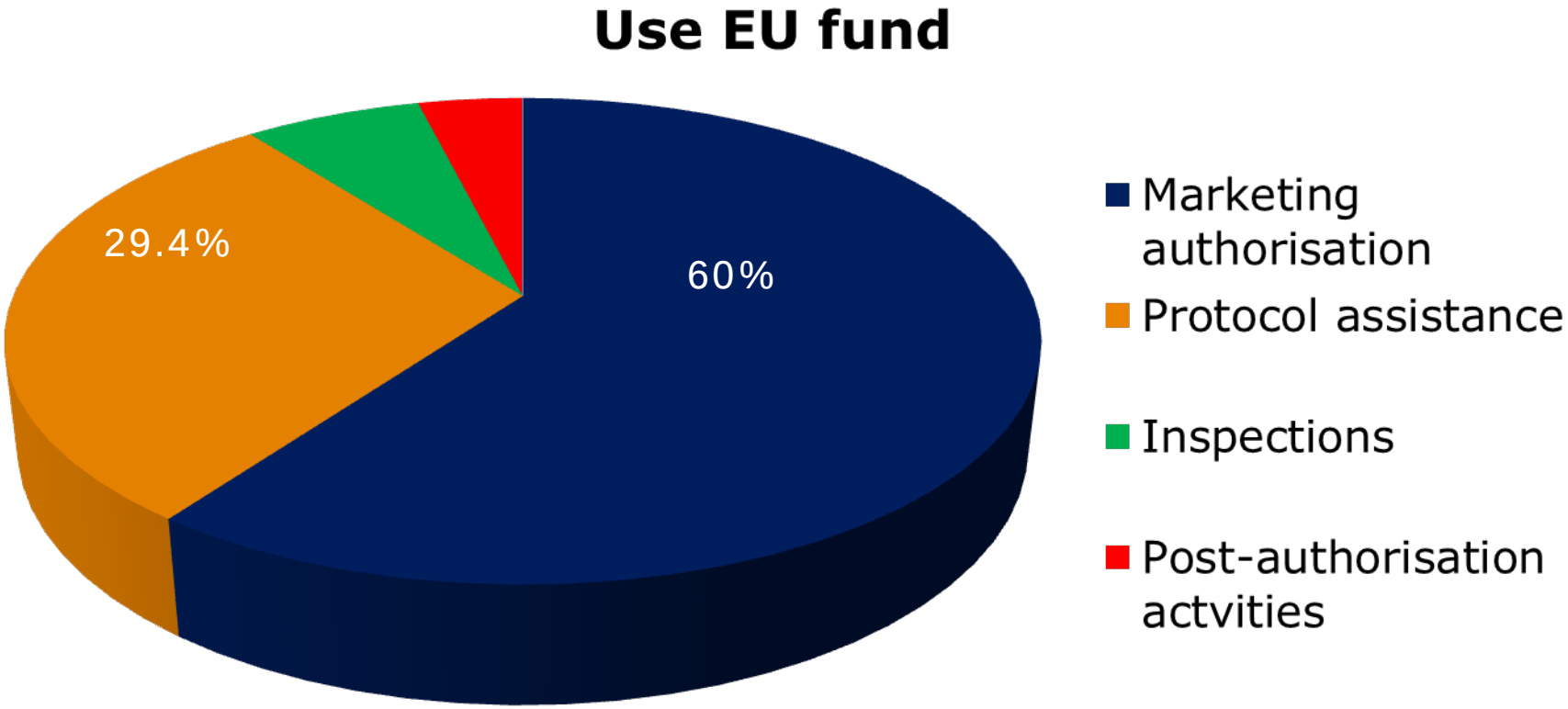
(+ 2 if paediatric indication – completion investigation plan)

- Protection against
  - similar products
    - Molecular structure
    - mech of action
    - for same indication
  - Three derogations (➔access to market even if similar)
    - Sponsor's consent
    - Lack of supply
    - Clinical superiority

Fee reductions

- Annually EU allocated special fund to cover fee reductions (approx. 6 million Euro)
- EMA has consistently kept maximum coverage for SMEs
- Academia and SME responsible for 79% development of advanced therapies
- Policy reviewed annually, needed revision in 2013 according to current budget

Allocation funds for fee reductions (2012)

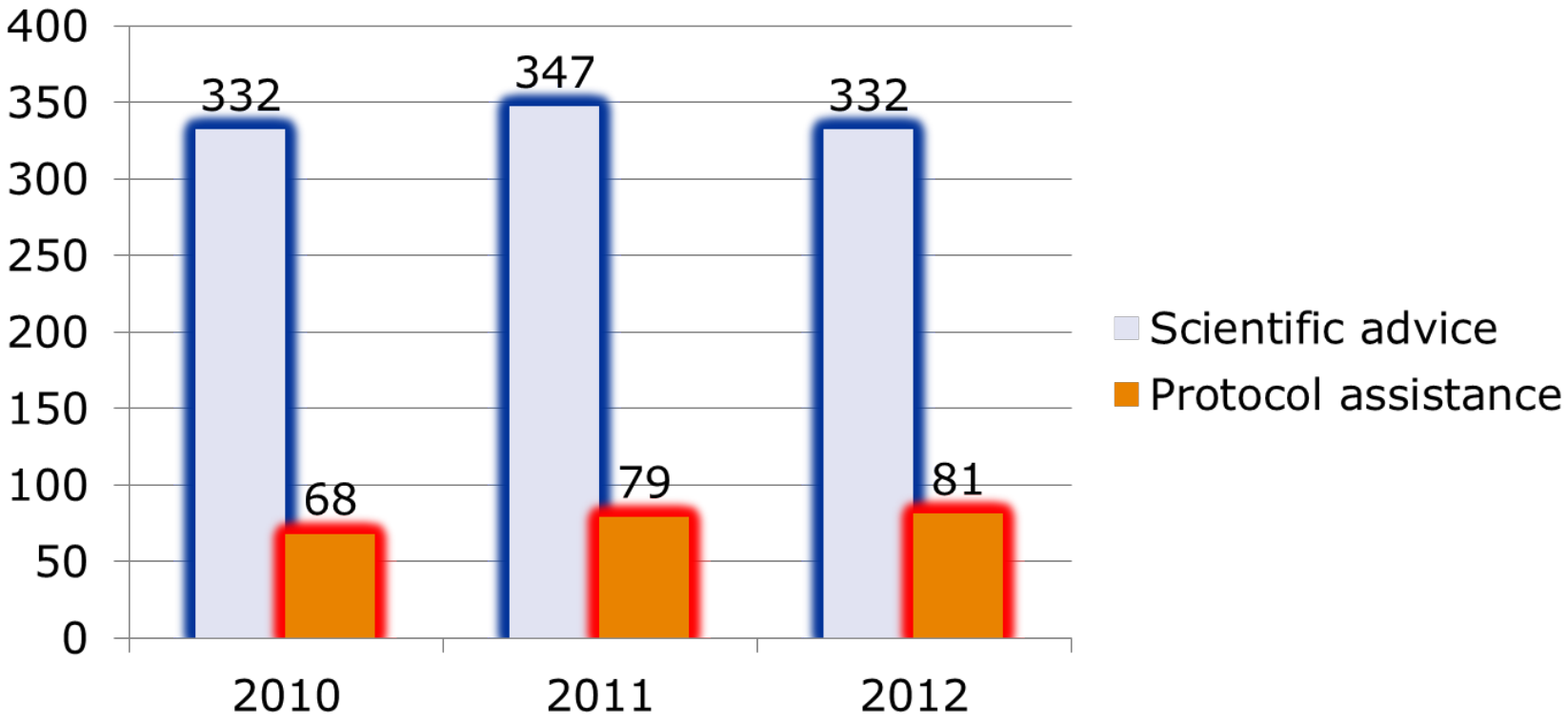


Protocol assistance

- Protocol assistance ≅ scientific advice
  - Questions on quality-efficacy-safety
  - Questions on significant benefit
  - Company position required
  - SAWP provides answers

  - CHMP adopts answers
  - COMP involved if issues on benefit

Protocol assistance



Designation criteria

RARITY (prevalence) / RETURN OF INVESTMENT

- Medical condition affecting not more than 5 in 10,000 in the EU (around 250,000 people)
- Without incentives it is unlikely that the marketing of the product would generate sufficient return to justify the necessary investment

SERIOUSNESS

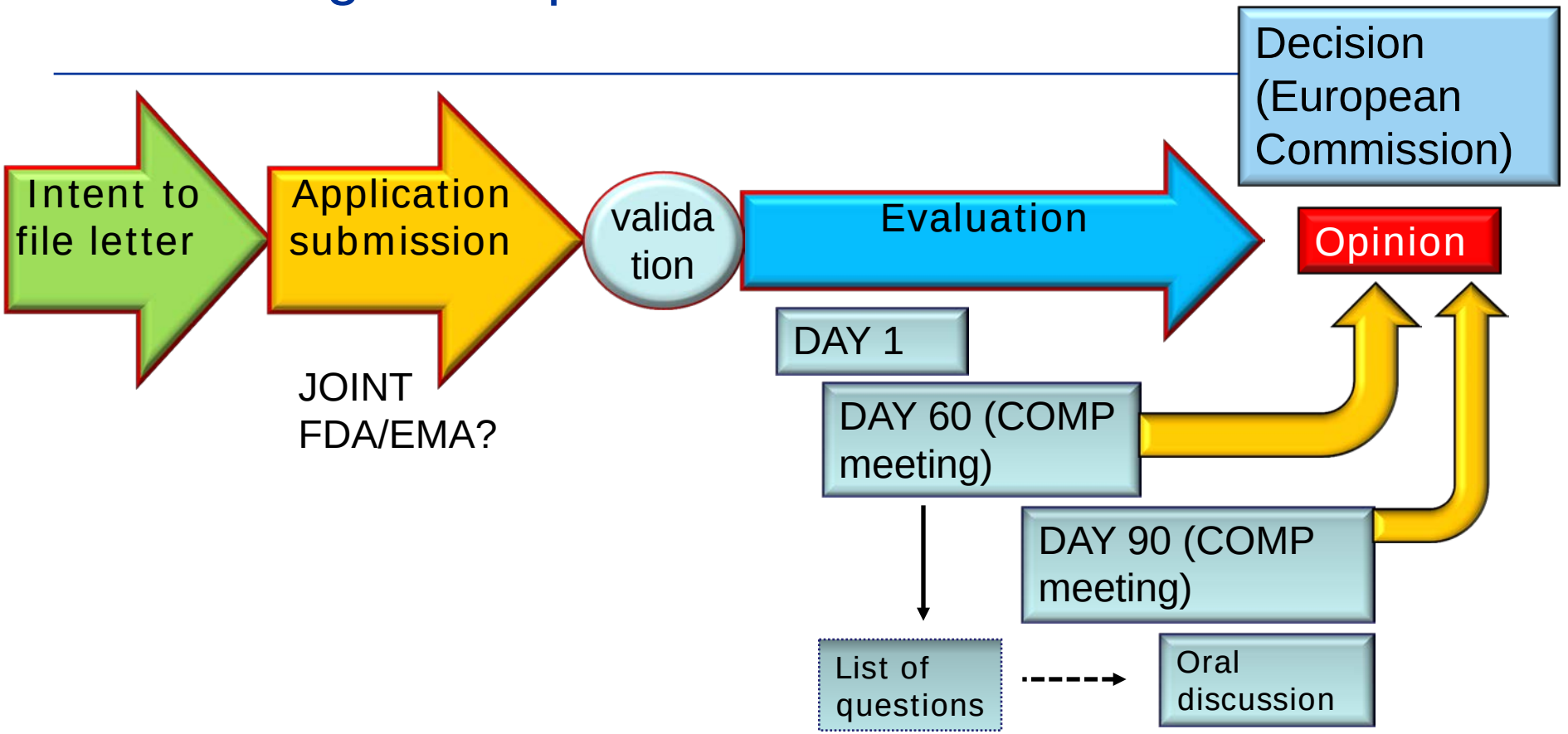
- Life –threatening or chronically debilitating

ALTERNATIVE METHODS AUTHORISED

EXCLUSIVE for EU

- If satisfactory method exist the sponsor should establish that the product will be of significant benefit

The designation process in the EU



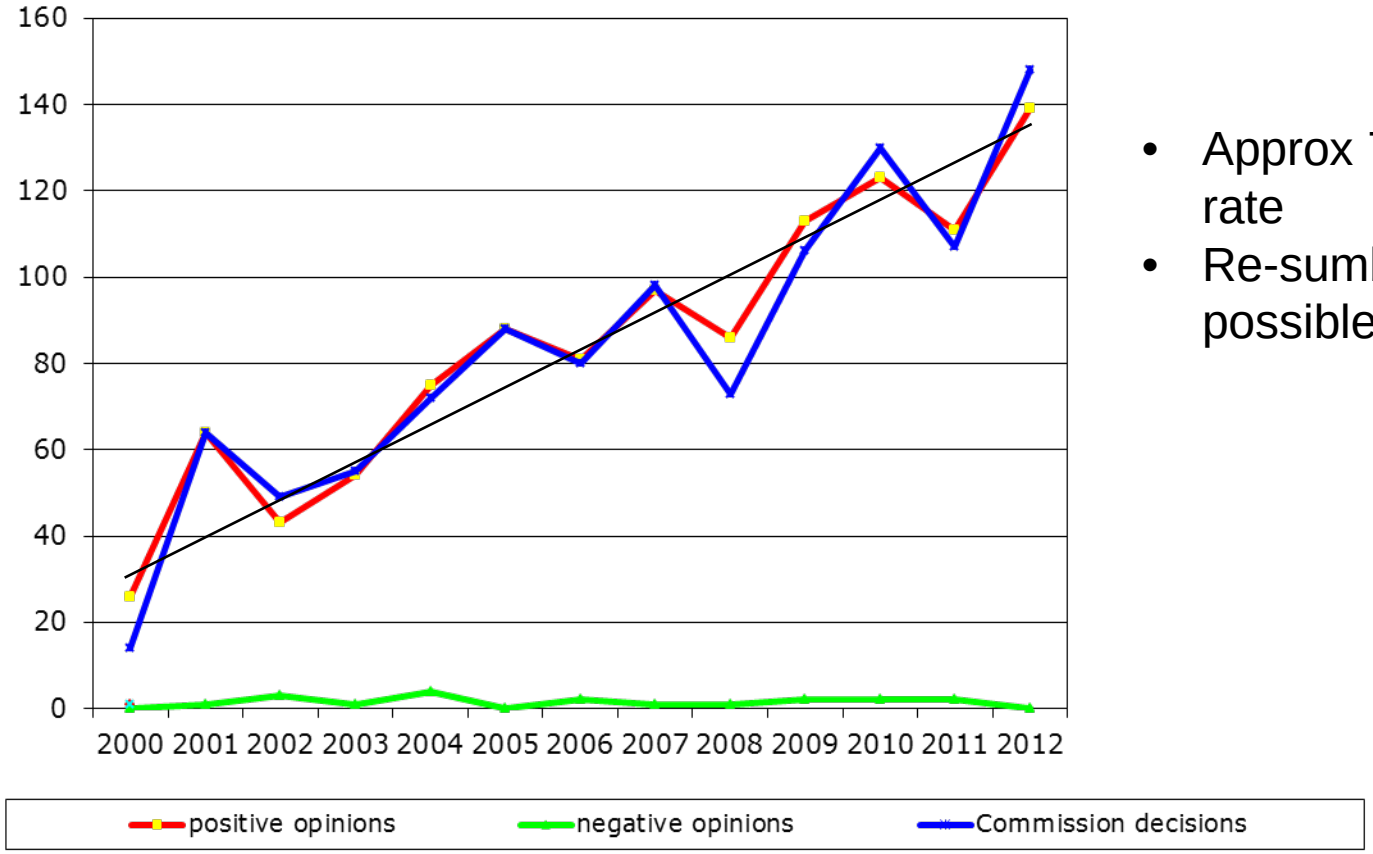
Committee for Orphan Medicines (COMP)

- 1 elected Chair (Prof Bruno Sepodes)
- 1 Representative per Member State
- 3 Patients' Representatives appointed by Eur. Commission
- 3 Members appointed by Eur. Commission on proposal from Agency
- 1 Member for Norway and 1 for Iceland

TOTAL: 33 members + 2 non voting

COMP responsibilities

Evolution orphan designations in the EU



- Approx 70% success rate
- Re-sumbmission possible and common

Five tips for your application

Tell us that you are coming (intent to file, email is enough)	
Use our templates	•application model (sections A to E) ; application form (EU and EMA/FDA)
Use our web resources	•Prevalence •Translations •National formularies
Use available guidance	•Format and content guideline •Application guidance
Ask for a pre-submission (email us!)	

Human medicines
- Pre-authorisation
- Post-opinion
- Post-authorisation
- Product information
- Scientific advice and protocol assistance
- Scientific guidelines
- Innovation Task Force
- Regulatory and procedural guidance
- SME office
- Paediatric medicine

Orphan designation
- Legal background
- How to apply
  - Submission deadlines

Home ▸ Regulatory ▸ Human medicines ▸ Orphan designation ▸ How to apply

How to apply for orphan designation

This page provides information for sponsors on how to apply for orphan designation for a medicine.

Notification of intention to submit

Sponsors should notify the European Medicines Agency of their **intention** to submit an application at least two months prior to the planned submission date by sending an e-mail to orphandrugs@ema.europa.eu. The notification should include:

- the active substance;
- the proposed orphan indication;
- the name and address of the sponsor;
- the planned submission date for the designation application.

Presubmission meetings

The Agency strongly encourages sponsors to request a **presubmission meeting** with the Agency prior to filing an application.

Presubmission meetings usually take place via teleconference, unless the sponsor has a strong preference to come to the Agency in person.

Where possible, sponsors should request a pre-submission meeting at least two months prior to filing. Presubmission meetings for orphan designation are free of charge.

Related information
- Orphan designation
- Medicines for rare diseases: background information

Contact point:
orphandrugs@ema.europa.eu

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- Pre-authorisation
- Post-opinion
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  - How to apply
    - Submission deadlines

Home ▸ Regulatory ▸ Human medicines ▸ Orphan designation ▸ How to apply ▸ Questions and answers

Questions and answers: Orphan-designation application

Email | Print | Help | Share

This page lists the questions that sponsors may have on applying for orphan designation.

If you have a question that is addressed here, forward your request to orphandrugs@ema.europa.eu.

Back to top

Expand all items in this list

Status of Orphan Marketing Authorisation Applications: 78 granted to date

Adopted positive opinion

- 1 awaiting decision

Ongoing applications in review process

- 27 applications in review process

Variations / Line Extensions in review process

- 3 applications in review process

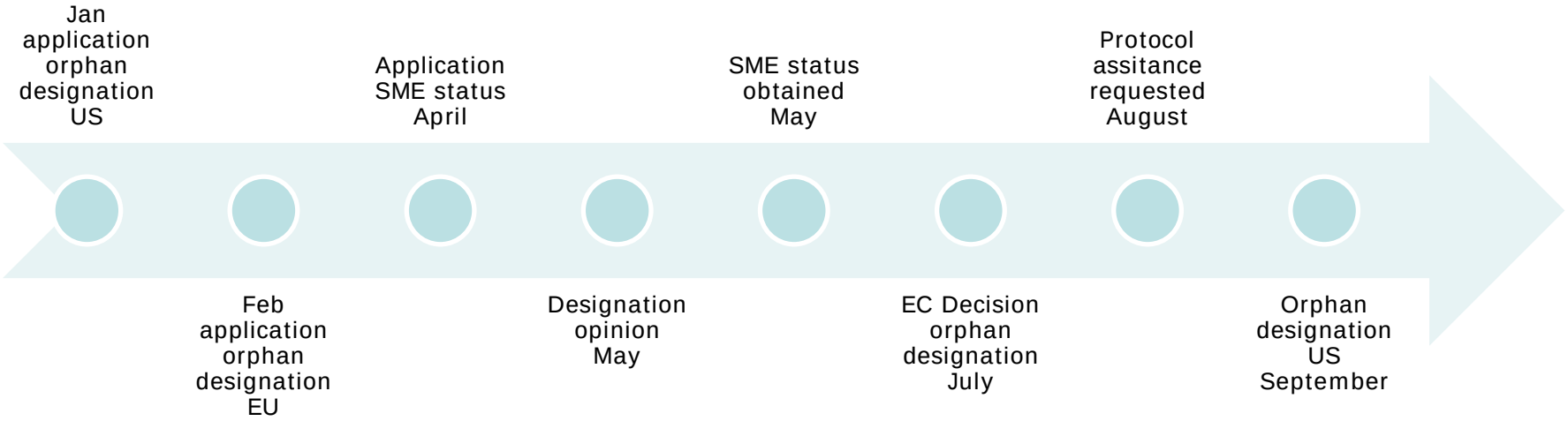
Negative outcomes for orphan MAA

- 56 applications withdrawn
- 10 negative decisions/refusals

Latest developments

- Strong EU and international support to research in rare diseases (Horizon 2020 and IRDiRC)
- Increasing international collaboration between regulators:
  - Administrative simplification / harmonisation
  - Continuous improvement (web linking, consultation on procedures)

Let's dream



Plan your strategy
Understand the step approach and use regulatory collaboration for your benefit

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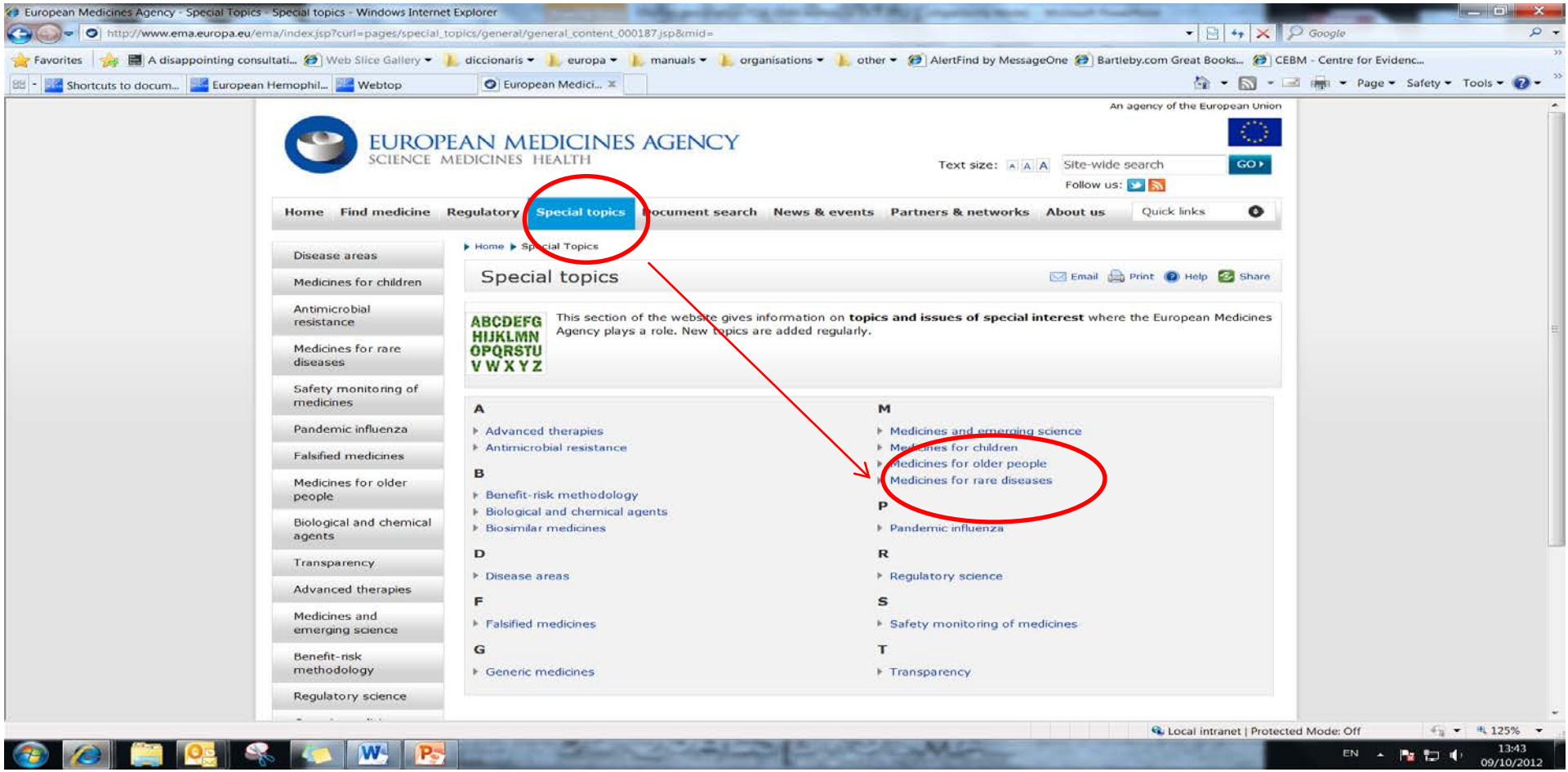
Of the 12 projects in the article nine for orphan designated products

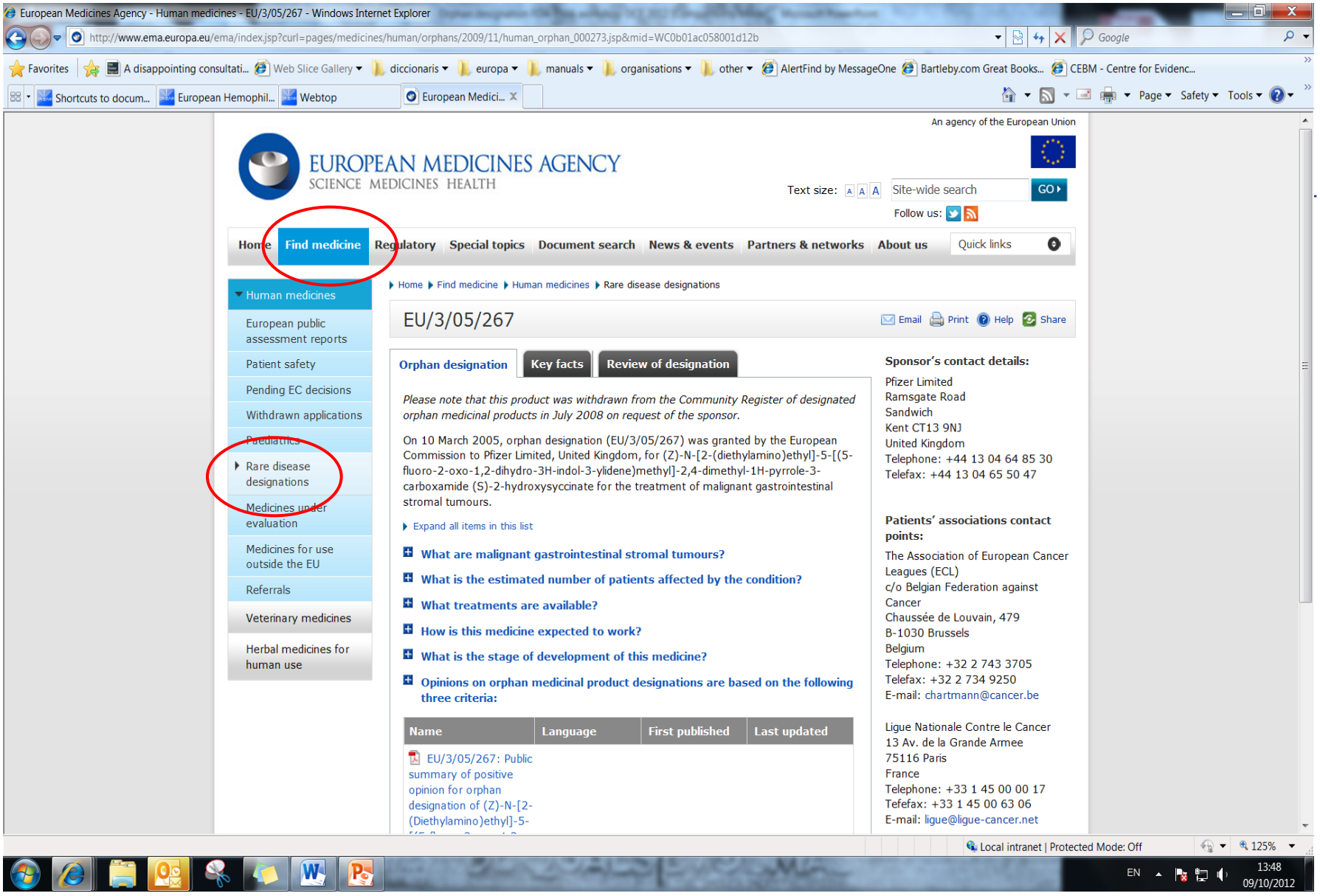
Cordis website: three additional projects granted that involve development of orphan drugs:

- Drugsford (inherited photoreceptor degeneration)
- Eurofancolen (fanconi anaemia)
- Aipgene (intermittent acute porfiria)

Rare disease project	Funding (million)
Neuromics: integrated European omics research project for diagnosis and therapy in rare neuromuscular and neurodegenerative diseases	€12
RD-CONNECT: an integrated platform connecting registries, biobanks and clinical bioinformatics for rare disease research	€12
EURenOmics: European Consortium for high-throughput research in rare kidney diseases	€12
BALANCE: development of a bioartificial liver therapy in acute liver failure	€6
DevelopAKUre: clinical development of nitisinone for alkaptonuria	€6
FIGHT-HLH: first targeted therapy to fight hemophagocytic lymphohistiocytosis	€6
GAPVAC: glioma actively personalized vaccine consortium	€6
MeuSIX: clinical trial of gene therapy for mucopolysaccharidosis type VI — a severe lysosomal storage disorder	€6
Net4CGD: gene therapy for X-linked chronic granulomatous disease	€6
PREVENTROP: new approach to treatment of the blinding disease retinopathy of prematurity	€6
PROFNAIT: development of a prophylactic treatment for the prevention of fetal/neonatal alloimmune thrombocytopaenia	€6
Traumakine: interferon-beta treatment of acute respiratory distress syndrome	€6

Where to have more information




European Medicines Agency - Human medicines - EU/3/05/267 - Windows Internet Explorer
http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2009/11/human_orphan_000273.jsp&mid=WC0b01ac058001d12b
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Webtop
European Medici...
An agency of the European Union
EUROPEAN MEDICINES AGENCY
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Quick links
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Pending EC decisions
Withdrawn applications
Paediatrics
Rare disease designations
Medicines under evaluation
Medicines for use outside the EU
Referrals
Veterinary medicines
Herbal medicines for human use
Home ▸ Find medicine ▸ Human medicines ▸ Rare disease designations
EU/3/05/267
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Help
Share
Orphan designation
Key facts
Review of designation
Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in July 2008 on request of the sponsor.
On 10 March 2005, orphan designation (EU/3/05/267) was granted by the European Commission to Pfizer Limited, United Kingdom, for (Z)-N-[2-(diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysyccinate for the treatment of malignant gastrointestinal stromal tumours.
Expand all items in this list
What are malignant gastrointestinal stromal tumours?
What is the estimated number of patients affected by the condition?
What treatments are available?
How is this medicine expected to work?
What is the stage of development of this medicine?
Opinions on orphan medicinal product designations are based on the following three criteria:
Name
Language
First published
Last updated
EU/3/05/267: Public summary of positive opinion for orphan designation of (Z)-N-[2-(Diethylamino)ethyl]-5-
Sponsor's contact details:
Pfizer Limited
Ramsgate Road
Sandwich
Kent CT13 9NJ
United Kingdom
Telephone: +44 13 04 64 85 30
Telefax: +44 13 04 65 50 47
Patients' associations contact points:
The Association of European Cancer Leagues (ECL)
c/o Belgian Federation against Cancer
Chaussée de Louvain, 479
B-1030 Brussels
Belgium
Telephone: +32 2 743 3705
Telefax: +32 2 734 9250
E-mail: chartmann@cancer.be
Ligue Nationale Contre le Cancer
13 Av. de la Grande Armee
75116 Paris
France
Telephone: +33 1 45 00 00 17
Tefefax: +33 1 45 00 63 06
E-mail: ligue@ligue-cancer.net
Local intranet | Protected Mode: Off
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09/10/2012

Many thanks

any questions?

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EMA website: http://www.ema.europa.eu