EMA FDA Gaucher's Disease Workshop

Opening remarks

September 17-18, 2012
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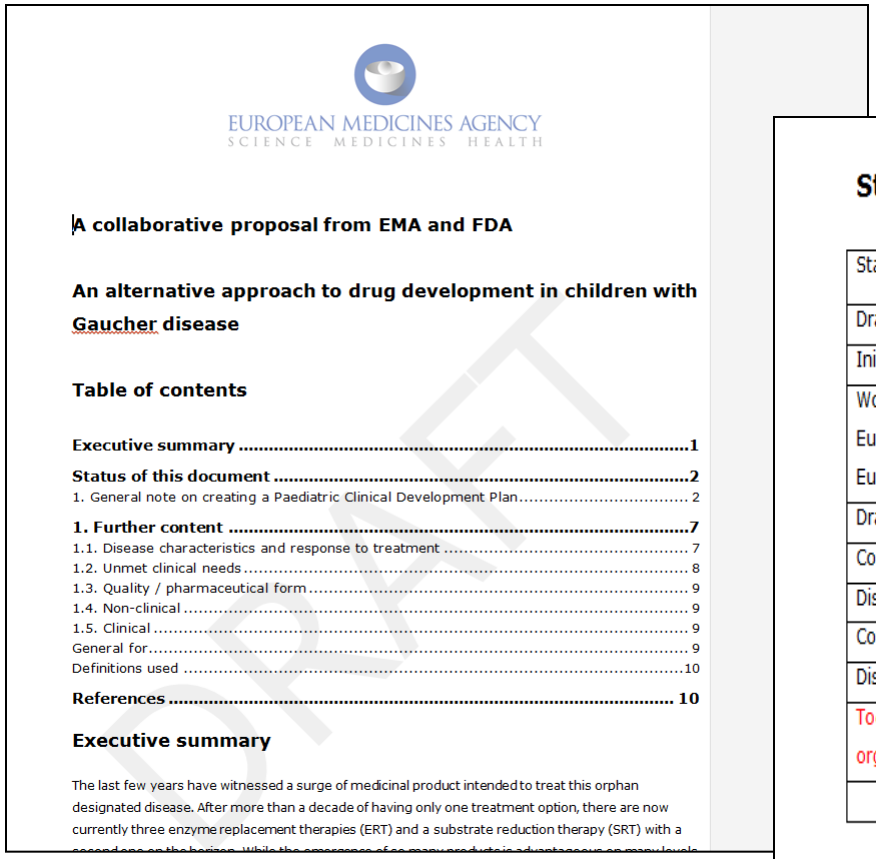
Division of Gastroenterology and Inborn Errors Products
CDER/FDA

Janusz Korczak (1878-1942), a children's advocate, he spoke of a Declaration of Children's Rights long before any such document was drawn up by the Geneva Convention (Korczak: 1924) or the United Nations General Assembly (Korczak: 1959)

A hundred children, a hundred individuals who are people – not people-to-be, not people of tomorrow, but people now, right now – today

How To Love A Child, Janusz Korczak.

Moving forward: Energy and Passion of Dr Davies



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

A collaborative proposal from EMA and FDA

An alternative approach to drug development in children with Gaucher disease

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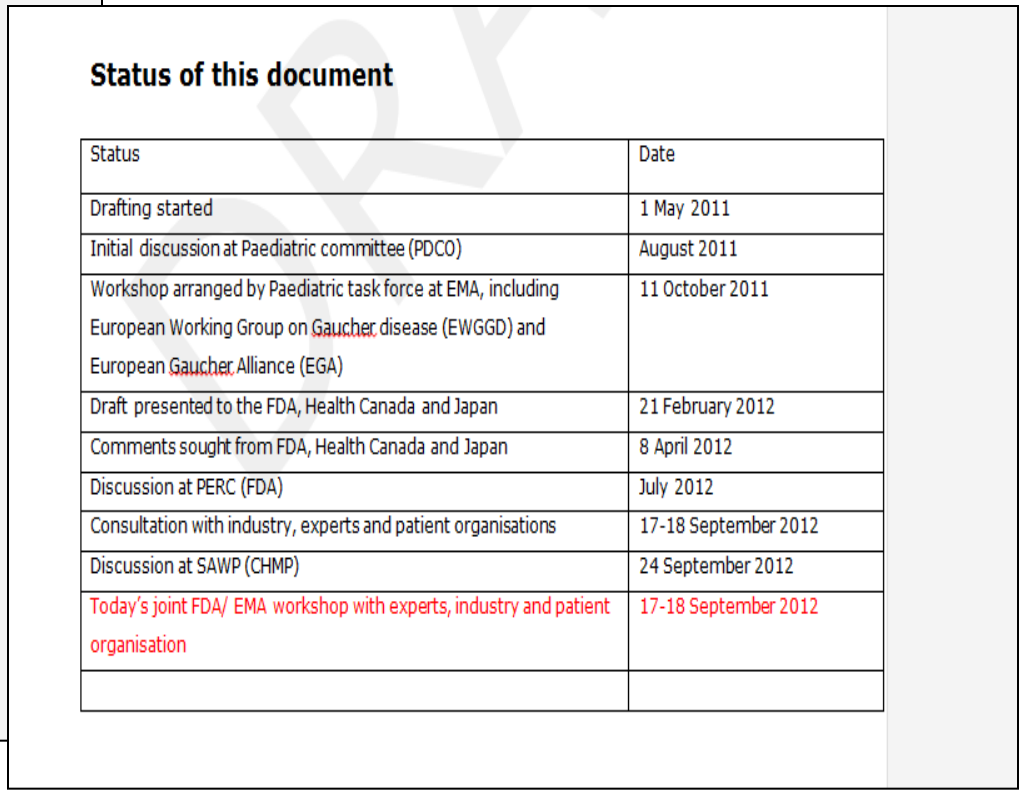
Executive summary

The last few years have witnessed a surge of medicinal product intended to treat this orphan designated disease. After more than a decade of having only one treatment option, there are now currently three enzyme replacement therapies (ERT) and a substrate reduction therapy (SRT) with a



Status of this document

Status	Date
Drafting started	1 May 2011
Initial discussion at Paediatric committee (PDCO)	August 2011
Workshop arranged by Paediatric task force at EMA, including European Working Group on Gaucher disease (EWGGD) and European Gaucher Alliance (EGA)	11 October 2011
Draft presented to the FDA, Health Canada and Japan	21 February 2012
Comments sought from FDA, Health Canada and Japan	8 April 2012
Discussion at PERC (FDA)	July 2012
Consultation with industry, experts and patient organisations	17-18 September 2012
Discussion at SAWP (CHMP)	24 September 2012
Today's joint FDA/ EMA workshop with experts, industry and patient organisation	17-18 September 2012

Dr Andrea Taft

- **Orphan Medicinal Products in the European centralised procedure – Current Marketing Authorisations for Gaucher Disease**

- **Analysis of Orphan Designation Decisions by EMA and FDA**
  - **92% …Same Decision**
  - **4%.....Different decision based on Prevalence differences**
  - **4%..... Different decision due to Different Analysis based on Laws and Regulations**

Source: Orphan-Drug Designations and Marketing Approvals in the New Millennium – The US and EU Experiences Source EURORDIS Round Table of Companies Workshop, 30 June 2006

- **Why are there Different Decisions?**
- **Different Laws**
  - **FDA**
    - **United States**
  - **EMA**
    - **European Union**

Critical Partnerships

- Academic Experts
- Patients and Families
- Industry
- Regulatory Partners

- We can make this work: We have already

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Partnership is the Key

- **“Coming together is a beginning; keeping together is progress; working together is success.”**
  Henry Ford

  http://www.brainyquote.com/quotes/authors/h/henry_ford.html

Back Up

- US FDA Criteria
  - 1. Prevalence …<200,000 people
  - 2. Data suggesting Efficacy
    - Human data or animal model of the specific disease. *In vitro* data may be used but uncommon
    - Clinical Superiority if already approved

- EMA Criteria
  - 1. Prevalence …Rate…<5/10,000 people (~<250,000)
  - 2. Efficacy including Clinical Superiority
  - ***3. Life Threatening or Chronically Debilitating, Serious Disease***
  - ***4. Are there other available treatment methods?…If so, require demonstration of significant benefit***

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