



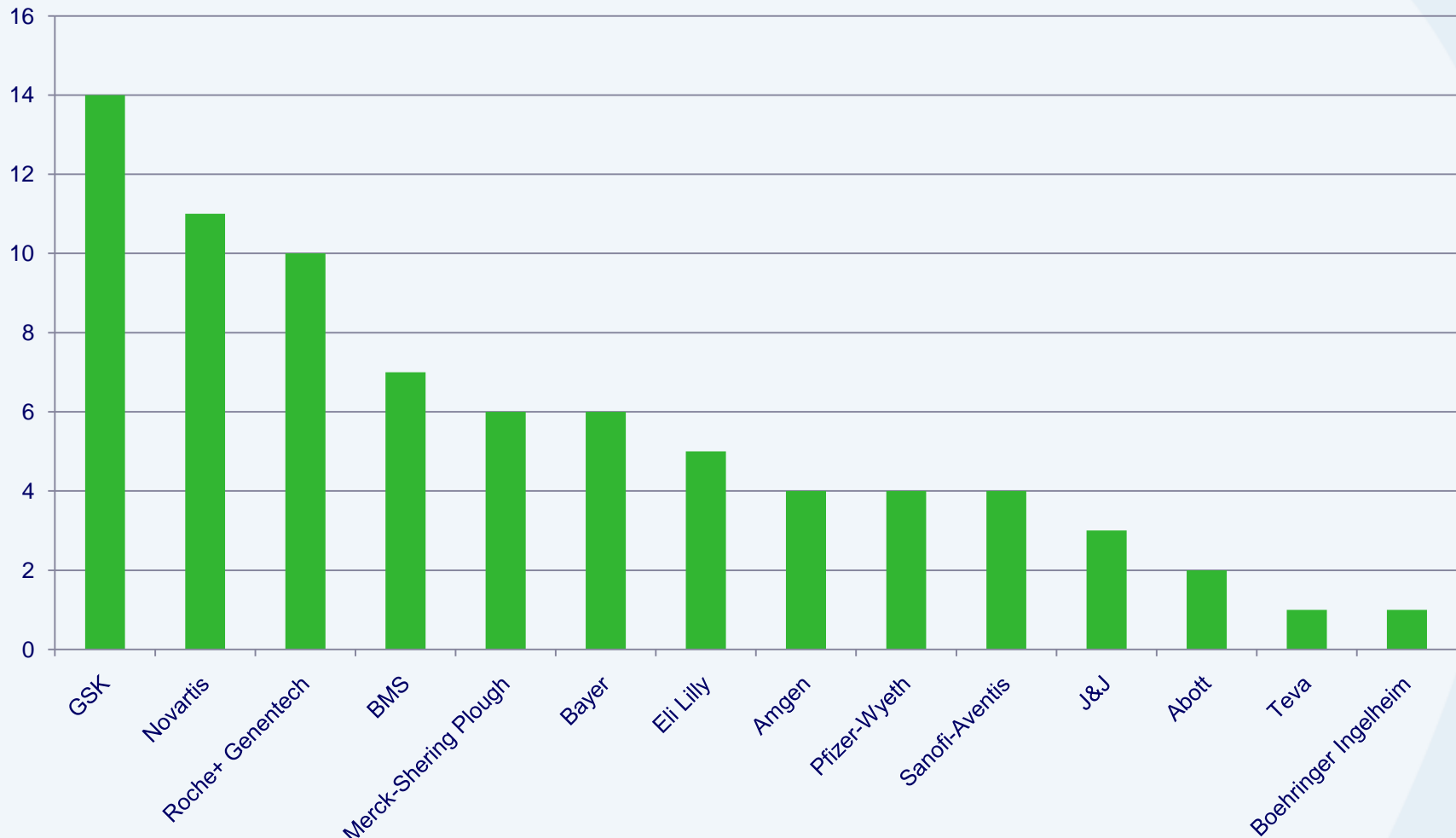
IT'S TIME TO STOP WASTING!

**8th International Annual
Duchenne Conference**

GSK Rare Diseases

GSK Rare Diseases - past

Drugs approved by FDA for orphan diseases



Source: FDA March 2010

GSK Rare Diseases - present

Medical need is overwhelming

- disabling
- life threatening
- inadequate treatments
- impact upon patients, upon families and on society

Rare diseases are not rare ... collectively

- 7,000 rare diseases
- 5% of the population
- less than 10% of patients receive treatment

New technologies offer hope

- Human Genome Project
- personalised medicines

Highly committed advocacy organisations

- **IT'S TIME TO STOP WASTING!**

GSK Rare Diseases - future

**Transform the lives of patients and their families
by developing medicines that will
prevent rare diseases or halt or reverse their progression**

GSK Rare Diseases - alliances

October 2010 – GSK and Amicus Therapeutics

- migalastat
- Fabry disease

October 2010 – GSK and Fondazione Telethon and Fondazione San Raffaele

- gene therapies
- SCID and eg. thalassaemia (via modified stem cells from bone marrow)

March 2010 – GSK and Isis Pharmaceuticals

- RNA therapeutics
- rare and infectious diseases

December 2009 – GSK and JCR Therapeutics

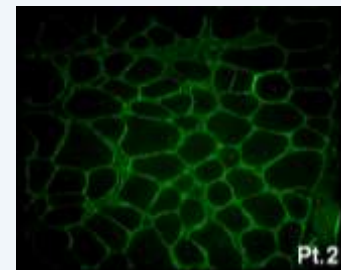
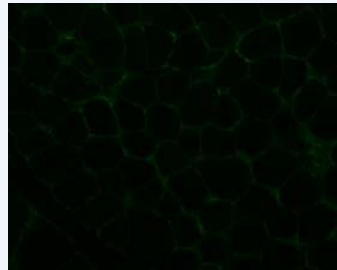
- enzyme replacement therapies
- Fabry disease and Hunter syndrome

Alliance to fight Duchenne Muscular Dystrophy

GSK and Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics, announced that they have entered into an exclusive worldwide collaboration for the development and commercialisation of RNA based therapeutics for Duchenne Muscular Dystrophy.

PRO-051

- Proof of mechanism
- Proof of concept



GSK2402968

- Phase II and III clinical trials for regulatory approval have commenced

GSK2402968

DMD114117

A Phase II double blind exploratory parallel group placebo controlled study to assess two dosing regimens of GSK2402968 for efficacy, safety, tolerability and pharmacokinetics in ambulant subjects with Duchenne muscular dystrophy

Current countries: Australia, Belgium, France, Germany, Netherlands, Spain, Turkey, UK

ORPHA236148

(www.orpha.net)

NCT01153932

(www.clinicaltrials.gov)

114117

(www.gsk-clinicalstudyregister.com)

GSK2402968

DMD114044

A Phase III randomised double blind placebo controlled study to assess the efficacy and safety of GSK2402968 in ambulant subjects with Duchenne muscular dystrophy

Current countries: Belgium, Canada, Chile, France, Germany, Italy, Japan, Korea, Netherlands, Poland, Russia, Taiwan

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(www.orpha.net)

NCTxxxxxxx

(www.clinicaltrials.gov)

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(www.gsk-clinicalstudyregister.com)

GSK2402968

DMD114118

A double blind escalating dose randomised placebo controlled study to assess pharmacokinetics, safety and tolerability in non-ambulant subjects with Duchenne muscular dystrophy

Current countries: USA

ORPHAxxxxxx

(www.orpha.net)

NCT01128855

(www.clinicaltrials.gov)

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(www.gsk-clinicalstudyregister.com)

GSK Rare Diseases

