

CLINIGEN

 *Global Access Programs*

PCMG meeting

Tom Watson, Business Development Director

January 2015



What is a Global Access Program?

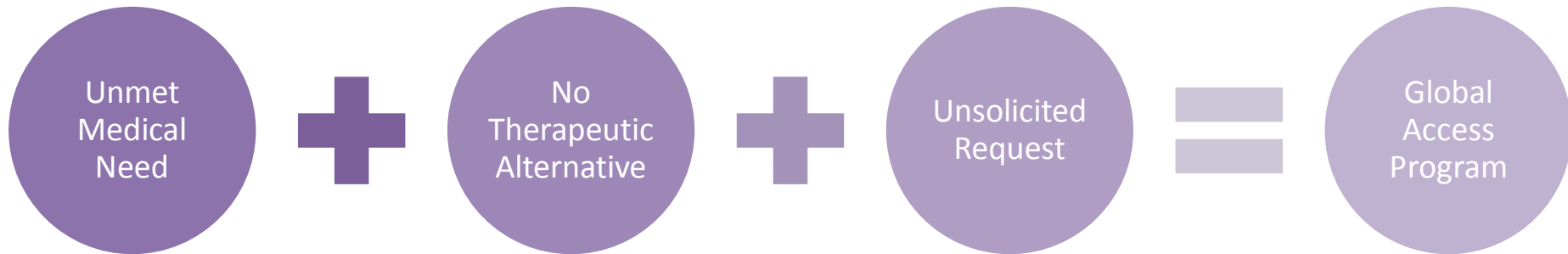
**A
mechanism that
enables patients with
an unmet medical need
to be provided with access
to a medicine, prior to
the medicine being
commercially available
in that country**

**EXPANDED ACCESS?
COMPASSIONATE USE?
EARLY ACCESS?
NAMED PATIENT PROGRAMS?
MANAGED ACCESS?**

Different Scenarios for Access Programs:

- Treatment is still in phase II or III development
- Treatment is approved by one agency such as the FDA but hasn't yet been approved by EMA or other regulators
- Product is withdrawn from the market but there remain patients in need of treatment
- Patients exiting clinical trials

Conditions for Early Access:



Orphan/Oncology/Rare disease drugs:

- High levels of unmet medical need
- Limited Treatment Options
- Innovative Products
- Pre-approval demand

Changing environment for Early Access

A growing need

Informed patients



Shared physician knowledge



Market access delays



Informed Patients

FOX NEWS [INSIDER]

MOST RECENT STORIES

- 13 MIN AGO Will Belgium's Legalized Euthanasia for Children Lead to Death Tourism?
- 1 HOUR 18 MIN AGO Sharyl Attkisson Resigns From CBS, Reportedly Due to Network's Liberal Bias
- 1 HOUR 43 MIN AGO Police Associations Sue NFL Over Gun Ban at Stadiums
- 3 HOURS 15 MIN AGO Drug Company Refuses 7-Year-Old Boy Life-Saving Medicine Despite Donations
- 12 HOURS 22 MIN AGO Ex-VP Cheney: Obama 'Hasn't Backed Up His Bold Rhetoric'
- 13 HOURS 22 MIN AGO Judge Nip Blasts De Blasio Over Plans to Shut Down Charter Schools
- 13 HOURS 46 MIN AGO Paul on Cruz: 'I'm Not Real Excited About Him Mischaracterizing My Views'
- 13 HOURS 54 MIN AGO Disney's Disney: Gerald Molen

COMMENT // SHARE

Company Denies Drug to 7-Year-Old Boy Struggling Against Curable Virus



BY FOX NEWS INSIDER // MAR 10 2014 // 9:34AM
AS SEEN ON FOX AND FRIENDS

Peter Johnson Jr. reported this morning on a harrowing story pitting the family of a seven-year-old Virginia boy against a drug company. Josh Hardy was stricken with an infection called an adenovirus after undergoing a bone marrow transplant in January.

The Boston Globe

Nation

NEWS METRO ARTS BUSINESS SPORTS OPINION LIFESTYLE MAGAZINE INSIDERS TODAY'S PAPER MY SAVED

Hopes of family, firm collide on unproven drug

By Tracy Zen

WASH. POST STAFF

JANUARY 31, 2014

ARTICLE COMMENTS (16)



Jamic and Jason Fowler with their children, Jack and Juliet. Jack was denied a possibly life-saving drug.

PRINT REPRINTS EMAIL SHARE

WASHINGTON — Jamie and Jason Fowler arrived early at a Chicago airport hotel on a recent Saturday morning, eager to meet the pharmaceutical executive from Massachusetts they hoped would help save the life of their 6-year-old son, Jack.

So began an encounter that provides a window into one of the most heart-wrenching dilemmas in modern medicine: should an experimental drug be given to a dying patient if it is unproven and might unravel a carefully designed clinical trial?

http://www.weitzlux.com/lawsuit/musculardyst Drug Shows Promise in Treatin... Experimental Drugs for Termin... Dad who pleaded for experime... Lawsuit

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Lawsuit For Muscular Dystrophy Drug

In This Section: [Malpractice News: Trial Muscular Dystrophy Drug](#)

Minnesota Family Files Lawsuit for Muscular Dystrophy Drug

A Minnesota family has filed a lawsuit against a New Jersey drug company to permit their son to participate in a clinical trial of an experimental drug to treat muscular dystrophy.

The 16 year old boy suffers from [Duchenne Muscular Dystrophy](#), a genetic and degenerative disease. Typically, patients diagnosed with this disease die in early adulthood because the disease causes deterioration and weakness in the muscles of the heart and lung.

The family alleges the company promised them their son would have access to the drug and the company discouraged them from enrolling him in a 2005 clinical trial. The family also alleges the company is citing their son's non-participation in the 2005 trial as one of the reasons for denying the boy treatment with the experimental drug.

The family has requested their son be permitted to participate through

Contact Us

Name:

Phone:

Email:

Case Description:

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VERDICTS & SETTLEMENTS

\$423 million settlement
MTBE suit involving the contamination of 153 public water systems nationally

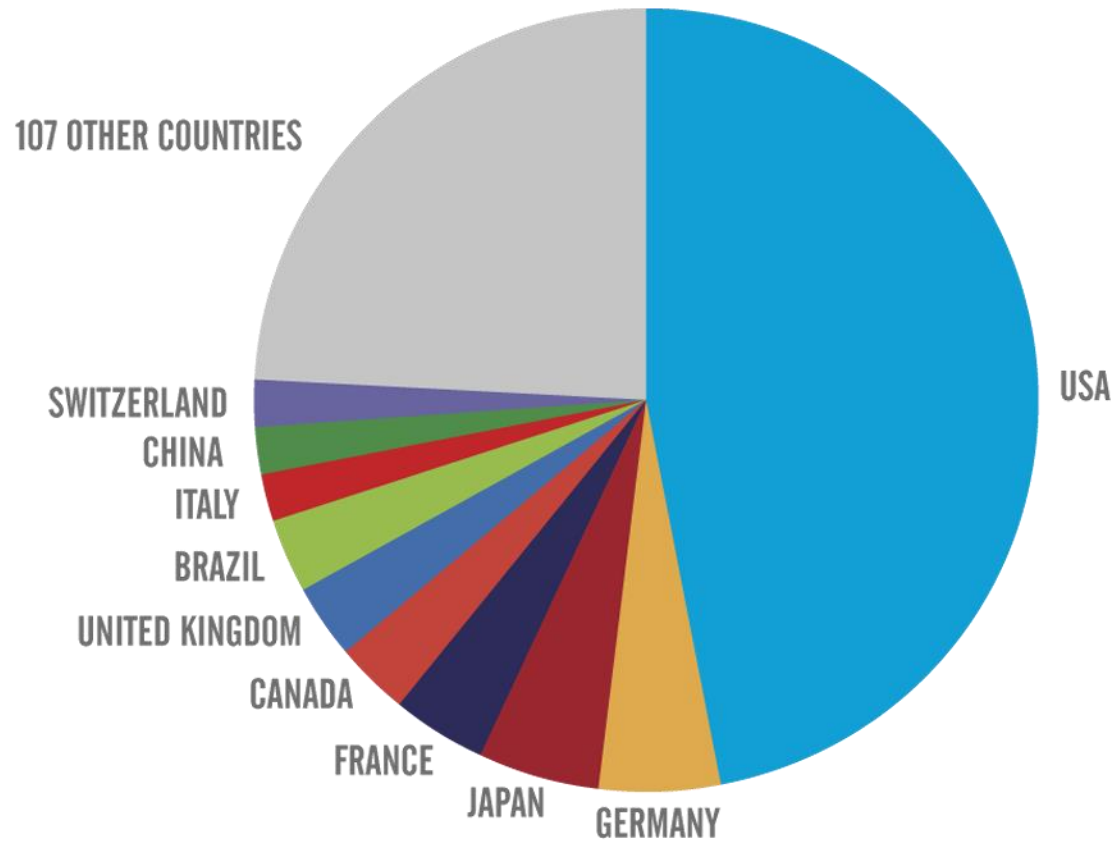
\$16.5 million verdict
Asbestos case involving exposure from dental tape

\$1.4 million settlement



Informed Physicians

25,500 professional oncology attendees – ASCO 2012



Data from American Society of Clinical Oncology www.asco.org

Benefits of Early Access

- **Ethically respond** to patient/physician requests
- **Accelerate access** to innovative products where treatment options are limited
- Developing and improving **clinical links** with medical community
- Capture **patient information** and 'real world' experience
- **Control** medicine supply, **manage risk** and set price precedent if necessary
- Maintain **supply chain integrity**, avoiding counterfeiting and international pharmacy trade
- Allow access to countries where commercialisation is not possible

There are distinct routes to access worldwide *Complex, dynamic regulatory environment*

U S A ...

74 FR 40942 13th August 2009

E U R O P E ...

Article 5 of Directive 2001/83/EC
Article 83 of Directive 2004/726/EC



E M E R G I N G M A R K E T S ...

Approval from Health Authority/ Personal Importation

Considerations for Global Access



CLINIGEN

Global Access Programs

