CLINIGEN

Global Access Programs

PCMG meeting

Tom Watson, Business Development Director

January 2015

Right Drug Right Patient Right Time

What is a Global Access Program?

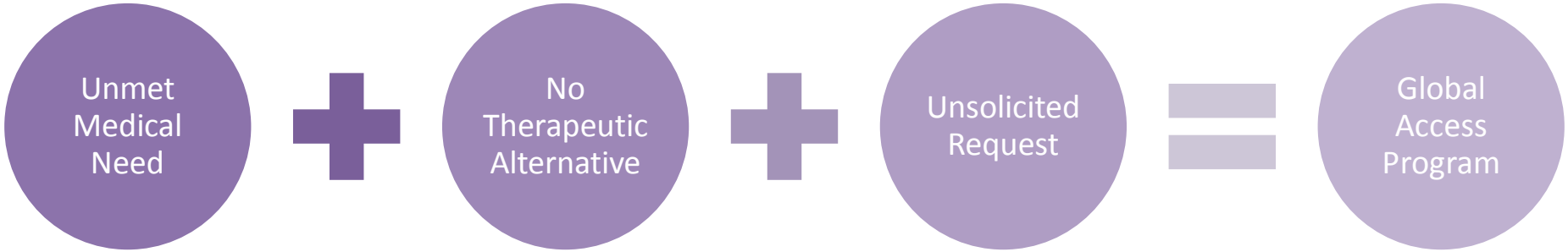
A mechanism that enables patients with an unmet medical need to be provided with access to a medicine, prior to the medicine being commercially available in that country

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- Product is withdrawn from the market but there remain patients in need of treatment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

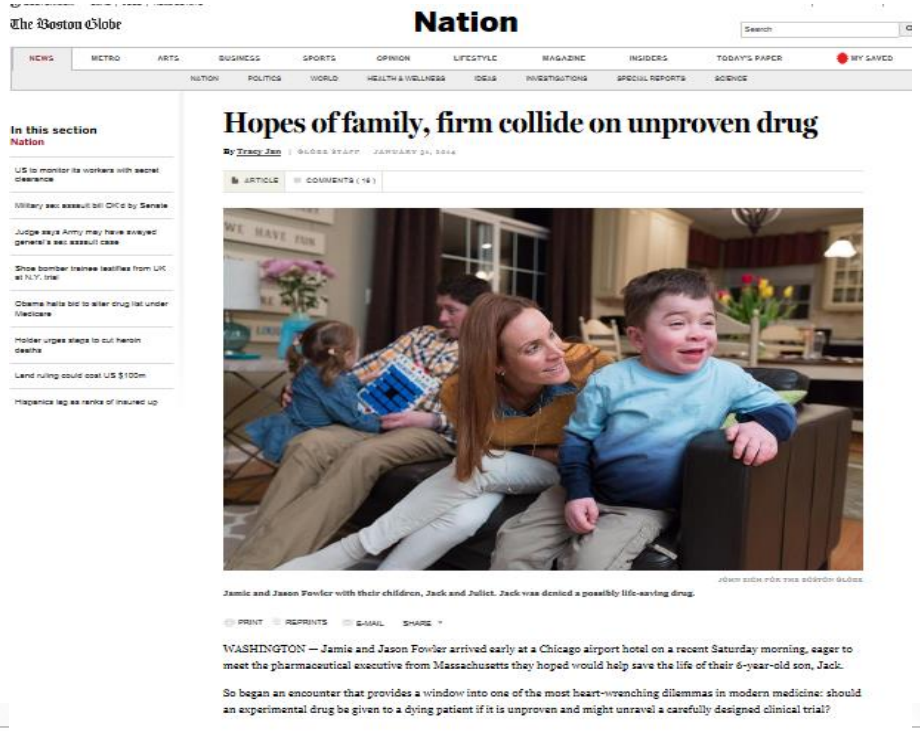
Changing environment for Early Access
A growing need

Informed patients

Shared physician knowledge

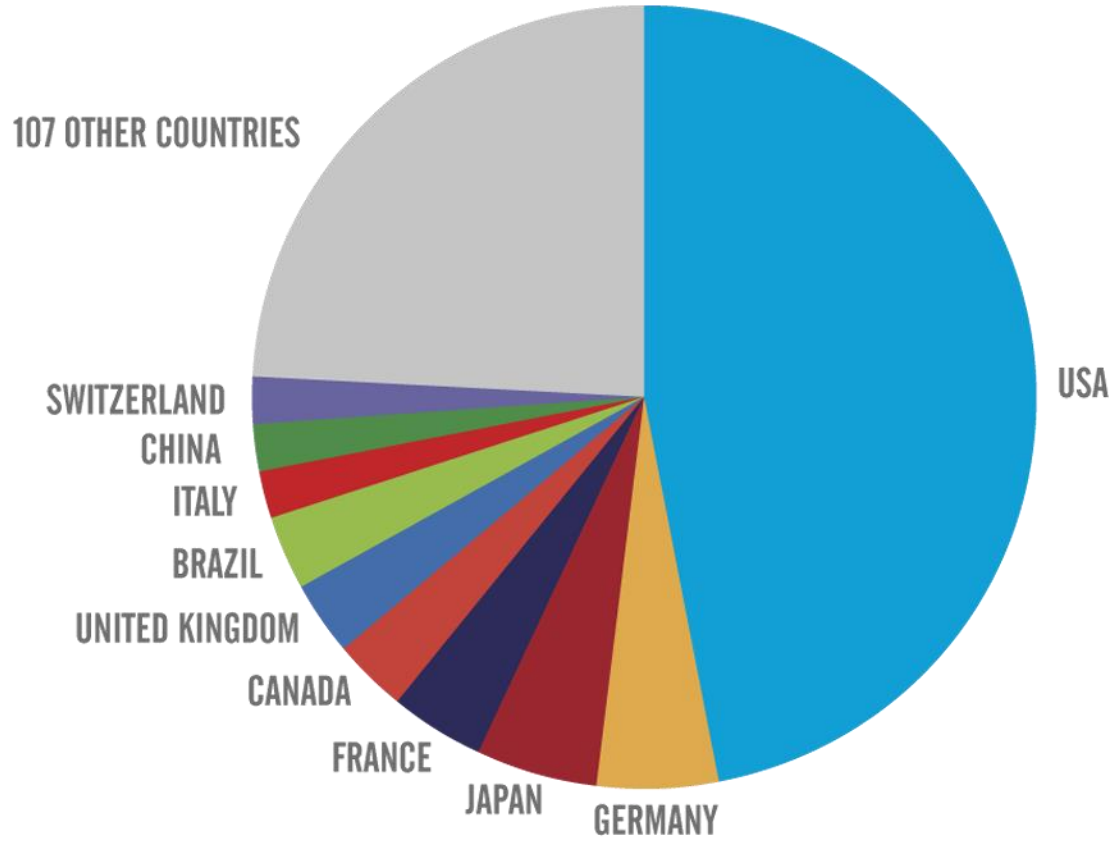
Market access delays

Informed Patients



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

Right Drug Right Patient Right Time