Putting **Rare** Diseases Patients First!

“The Patient’s Perspective”

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Founder and President
Putting Rare Diseases Patients First!

ASCPT 2016

Outline

- The World of the Rare Diseases Patient
- Patient Engagement and Empowerment
- Making an Impact: Your Role as Researchers and Drug Developers

The World of the Rare Diseases Patient

The World of the Rare Diseases Patient

Diagnosis

The World of the Rare Diseases Patient

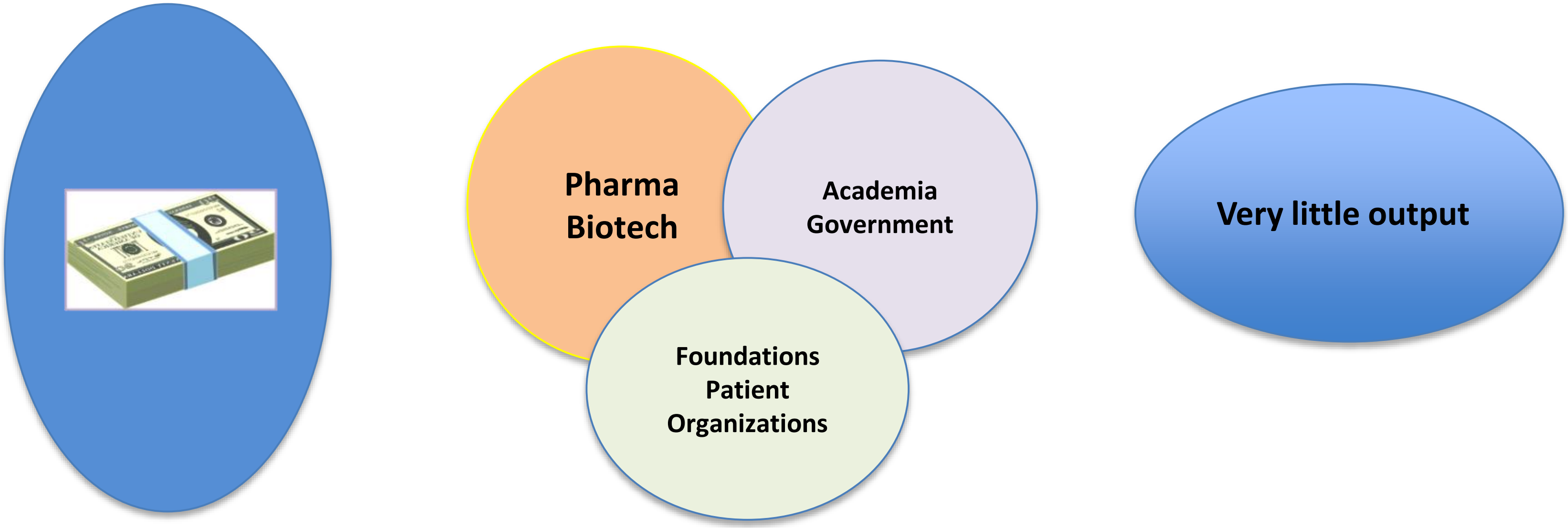
Treatment

The World of the Rare Diseases Parent

The World of the Rare Diseases Patient

Help

Help



Pharma
Biotech

Academia
Government

Foundations
Patient
Organizations

Very little output

EGAN
Patients Network for Medical Research and Health

EURORDIS
Rare Diseases Europe

ELPA
European Liver Patients Association

ENFA
European Network of Fibromyalgia Associations

EHC

Pain Alliance Europe

ehn

epha

SMA europe

We care, we share ICCCPO

debra
Working for a life free of pain

EFA
European Federation of Allergy and Airways Diseases Patients Associations

Genetic Alliance

RareConnect.org
A EURORDIS INITIATIVE

EPF
European Patients Forum

MPE
MYELOMA PATIENTS EUROPE

Alzheimer Europe

EFCNI
european foundation for the care of newborn infants

International Alliance of Patients' Organizations
A global voice for patients
Full Member

EGA
European Gaucher Alliance
Driven by passion for patients

TIF
THALASSAEMIA INTERNATIONAL FEDERATION

RareConnect.org
A EURORDIS INITIATIVE

IDDT
Insulin Dependent Diabetes Trust

EIWH

International Diabetes Federation

EUROPA UOMO

FasterCures
THE CENTER FOR ACCELERATING MEDICAL SOLUTIONS

emsp
EUROPEAN MULTIPLE SCLEROSIS PLATFORM

GAMIAN
EUROPE

IPOPI

EATG
European AIDS Treatment Group

NORD
National Organization for Rare Disorders

AGE
Platform Europe

FABRY
International Network

hai
EUROPE

BEUC
The European Consumer Organisation

EPDA
EUROPEAN PARKINSON'S DISEASE ASSOCIATION

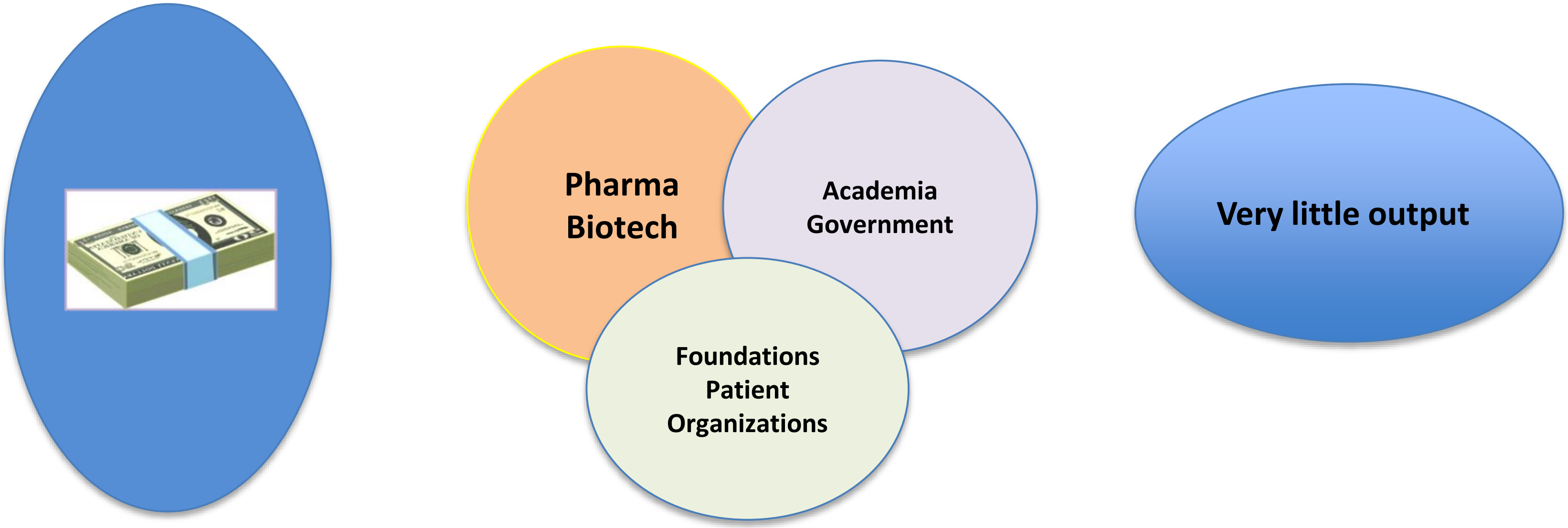
EFNA
European Federation of Neurological Associations

IBE

ELF
EUROPEAN LUNG FOUNDATION

EUROPEAN CANCER PATIENT COALITION

Help



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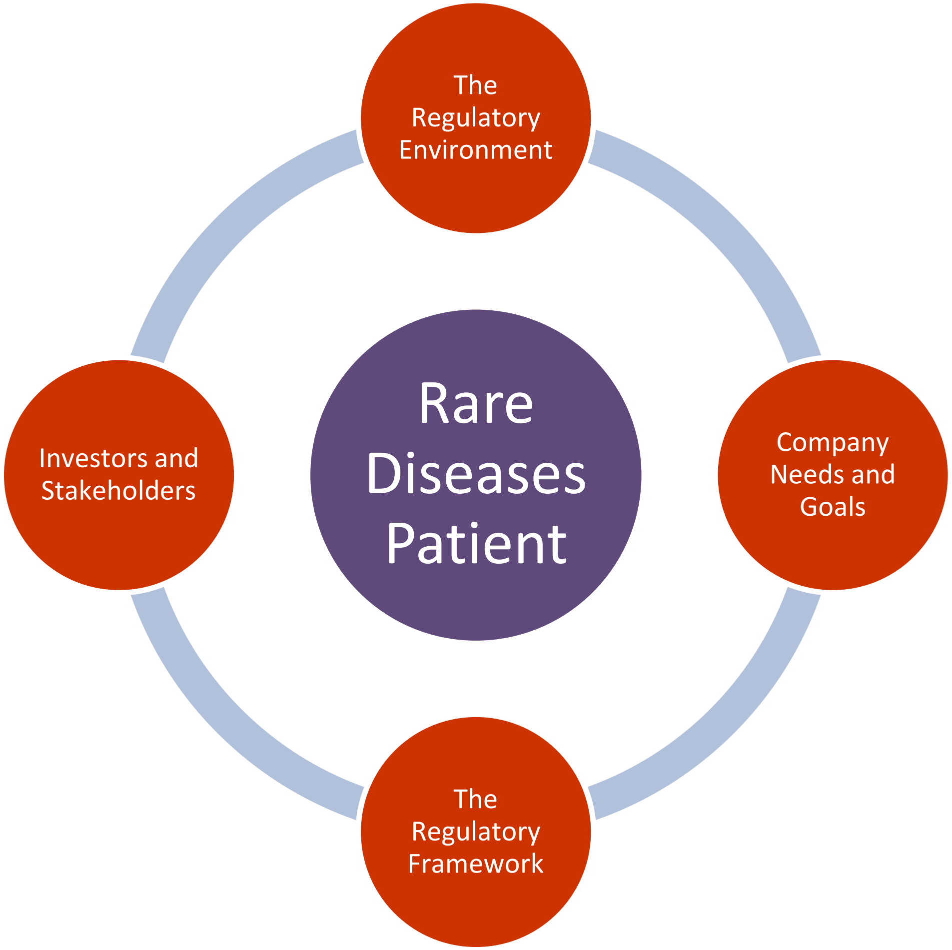
Patient Engagement and Empowerment

Engagement and Empowerment

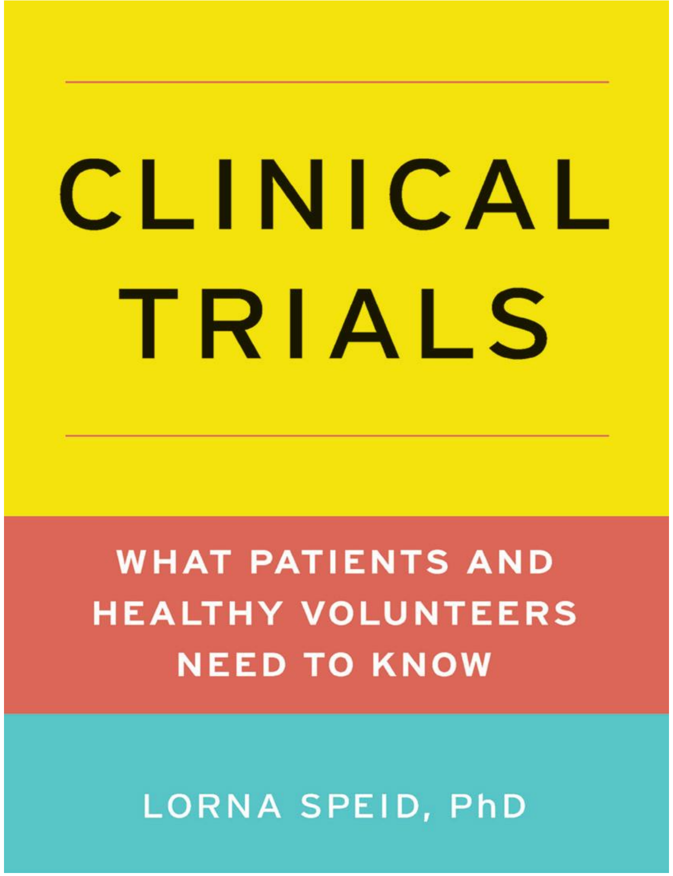


Patient Empowerment

Putting Rare Diseases Patients First!

- PRDPF! Formed in January 2014
- 501 (c) (3) organization, charity status
- Tax exempt status in California and from the IRS
- Global outreach
- Free educational content
  - **Webinar 001** - ***The Seven Things You Need to Know about the Drug Development Process For Rare Diseases, Dr. Lorna Speid, PRDPF!***
  - **Webinar 002 - *“Will Someone Please Tell Me What is Wrong With Me?” – Dr. Timothy McDaniel, now with TGEN***
  - **Webinar 003** - ***“The Rare Diseases Patient at the Center of it All” – Dr. John Whyte, FDA***
  - **Webinar 004** – **“The Rare Diseases Patient at the Center of it All – Part 2”– Patient Engagement – Dr. Sangeetha Jethwa, Roche**
  - **Webinar 005 - *“Natural History Studies – Making a Contribution” - Part 1 – Laying a Foundation - Dr. Nuria Carrillo, NIH-NCATS***
  - **Webinar 006** – “***Natural History Studies and Your Rare Disease Child” – Part 2 –Dr. Ann Barbier, Agios Pharmaceuticals and Professor Morton Cowan, UCSF***

Patient Empowerment



CLINICAL TRIALS

WHAT PATIENTS AND HEALTHY VOLUNTEERS NEED TO KNOW

LORNA SPEID, PhD

PRDPF! Facebook page:

https://www.facebook.com/puttingrarediseasespatientsfirst

PRDPF! Twitter:

https://twitter.com/PuttingRDPF

PRDPF! Blog:

https://rarediseases123.wordpress.com/

Engagement: Risk Benefit Assessment

Engagement: Risk Benefit Assessments

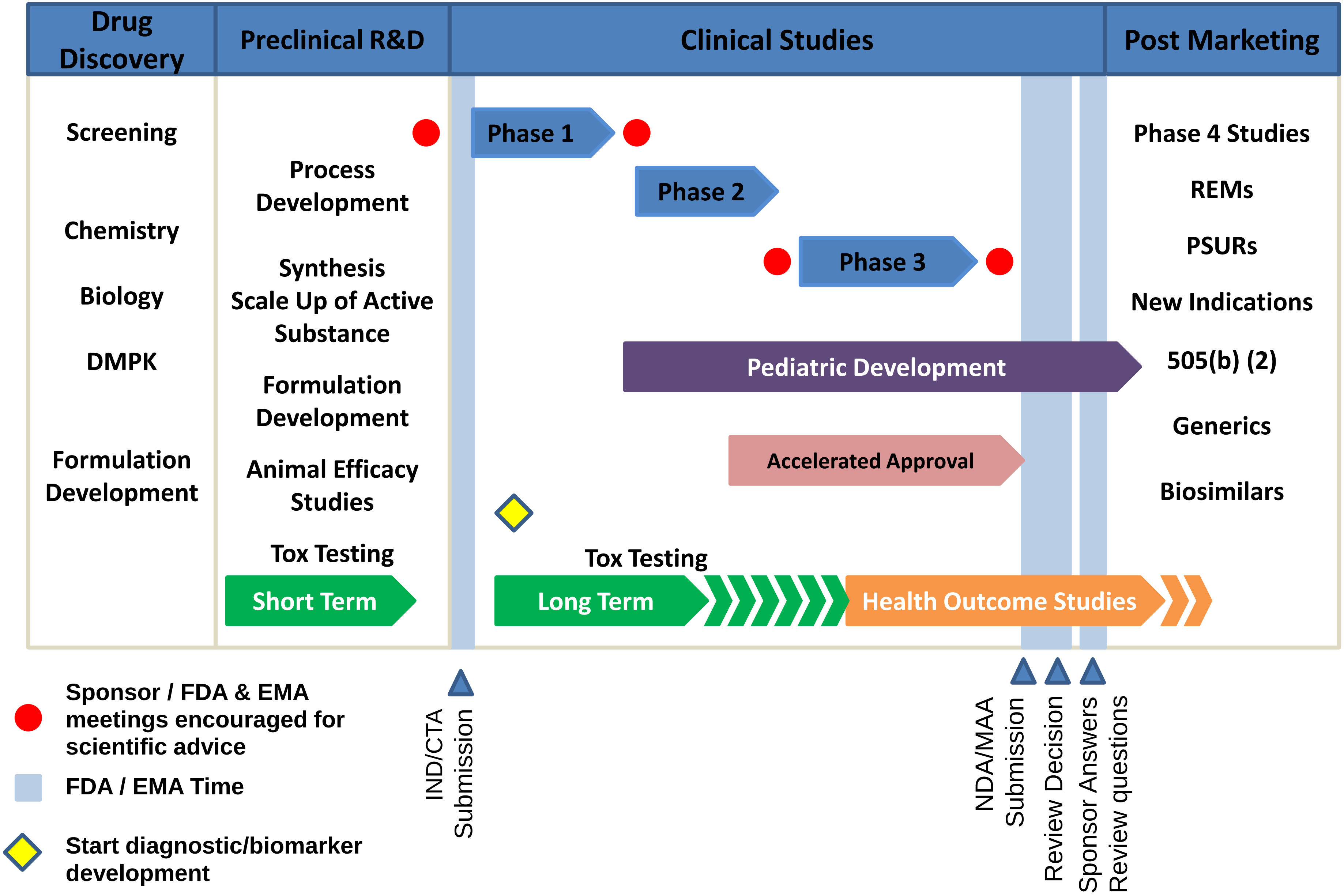
Patient Scenarios

Adult male or female
Recent diagnosis
No known treatment
No natural history studies
Rapidly progressing
Shortened lifespan

Adult patient
Diagnosed some years ago
Treatment is suboptimal or only treats symptoms
Disease is slowly progressing
Lifespan may or may not be shortened
Natural history of the disease is known

The disease afflicts children
Numbers of children impacted are small
No effective treatments
Rapidly progressing
Shortened lifespan

Making an Impact



Drug Discovery	Preclinical R&D	Clinical Studies	Post Marketing
Screening Chemistry Biology DMPK Formulation Development	Process Development Synthesis Scale Up of Active Substance Formulation Development Animal Efficacy Studies Tox Testing Short Term	Phase 1 Phase 2 Phase 3 Pediatric Development Accelerated Approval Tox Testing Long Term Health Outcome Studies	Phase 4 Studies REMs PSURs New Indications 505(b) (2) Generics Biosimilars

IND/CTA Submission

NDA/MAA Submission

Review Decision

Sponsor Answers Review questions

● Sponsor / FDA & EMA meetings encouraged for scientific advice

▨ FDA / EMA Time

◆ Start diagnostic/biomarker development

Patient Involvement

Pharma, Biotech, and Academia Engaging with Patients

- Engage with Patients
  - Targeted Product Profile
  - Global Regulatory Strategy – *Use regulatory mechanisms to speed up access to commercialization [patients]*
  - Well designed clinical trials
  - Well designed development programs
  - Right patients – Representative
  - Using the technologies available to us – right sized studies
  - Re-engineering our industry
- Reimbursement
- Get it Right First Time – Fastest Time to Market

Patient Involvement – What Does it Look Like?

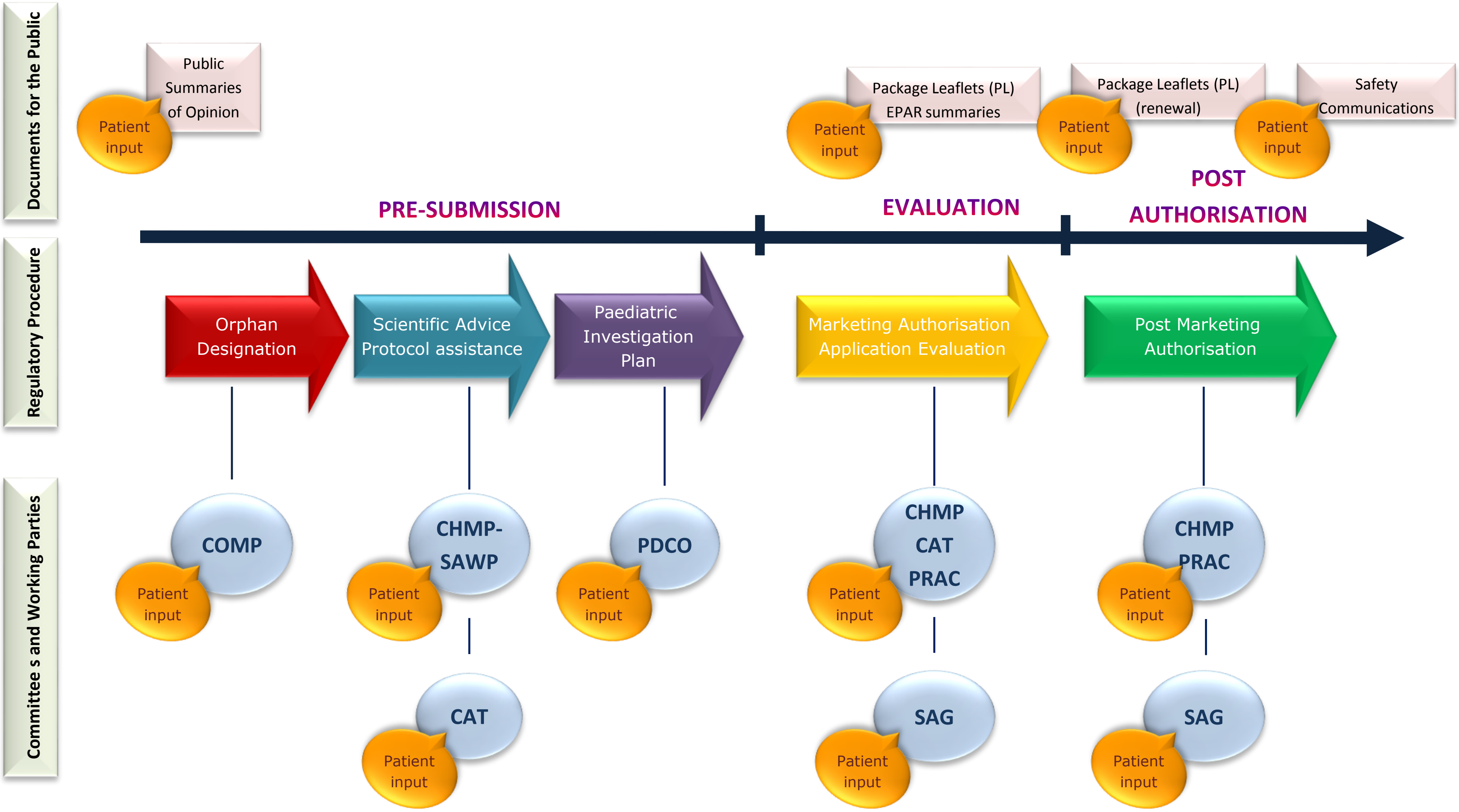
- Risk benefit assessment programs
- Natural History Studies – design and participation
- Patient Registries
- Legislation
- Regulatory Authorities
- Contribution to the design of clinical trials
- Ensuring there is sufficient support for patients in the trenches

Regulatory Environment

- FDA www.fda.gov
  - Patient Liaison Program
  - Patient Network Website
  - Patient Reported Outcome
  - Patient Focused Drug Development Meetings

Regulatory Environment

www.ema.Europa.eu



Documents for the Public

Regulatory Procedure

Committee s and Working Parties

Public Summaries of Opinion

Patient input

Package Leaflets (PL) EPAR summaries

Patient input

Package Leaflets (PL) (renewal)

Patient input

Safety Communications

Patient input

PRE-SUBMISSION

EVALUATION

POST AUTHORISATION

Orphan Designation

Scientific Advice Protocol assistance

Paediatric Investigation Plan

Marketing Authorisation Application Evaluation

Post Marketing Authorisation

COMP

Patient input

CHMP-SAWP

Patient input

CAT

Patient input

PDCO

Patient input

CHMP CAT PRAC

Patient input

SAG

Patient input

CHMP PRAC

Patient input

SAG

Patient input

Guidance for Best Practices

- No guidelines from FDA or EMA at this time
  - Be compliant with guidelines – when they are issued
- Care
  - Conflicts of interest
  - Vulnerability
  - Representative patients
  - Biases
- Templates
  - Don’t reinvent the wheel
  - Learn from other disease areas
  - Engage with organizations that are already going through the process
  - Speak to real patients and parents of children with the disease in question
- Specially Trained Personnel
  - Pharmacists
  - Physicians
  - Nurses

Dr. Lorna Speid

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