



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



European
Reference
Network

for rare or low prevalence
complex diseases

 Network
Bone Disorders (ERN BOND)

**Stakeholder perspectives on the development
of new methodologies**

BOND ERN perspective

Luca Sangiorgi MD, PhD

BOND ERN coordinator

***EMA/EC multi-stakeholder workshop to further improve the implementation
of the paediatric regulation
London, 20 March 2018***

An ERN-BOND White Paper On Diagnosis

ERN-BOND White Paper on
Diagnosis of Osteogenesis
Imperfecta: Synopsis



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Meena Balasubramanian, Valerie Cormier-Daire, WG
“Diagnostic Challenge”, ERN BOND

Inês Alves, ERN BOND EPAG, ALPE Foundation





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BOND ERN Working Group on Clinical Trial

March 2017

May 2018

Scoping

Patient groups

Investigators

Industry

Core data
definition

Primary projects

Secondary projects

Engagement

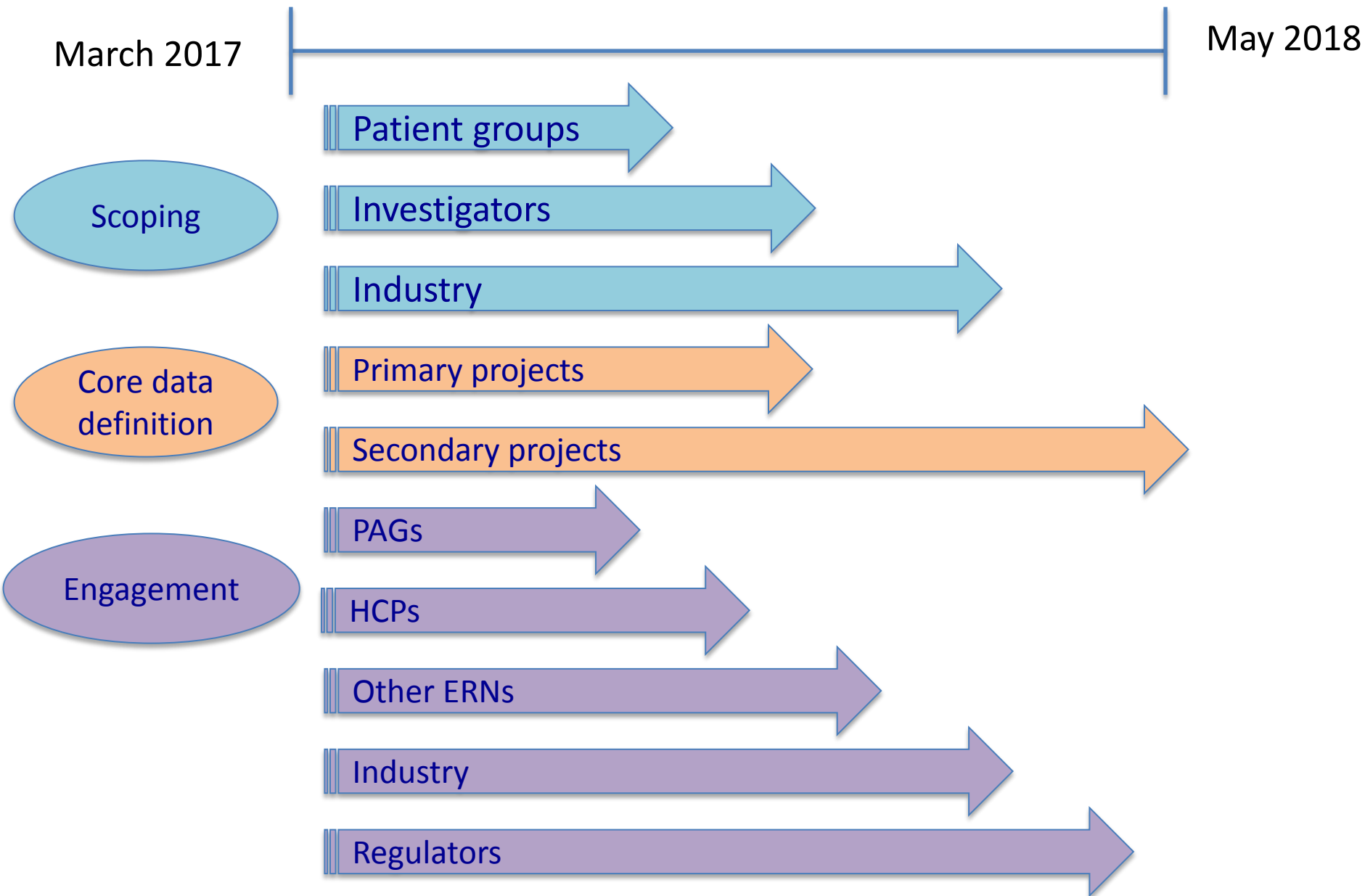
PAGs

HCPs

Other ERNs

Industry

Regulators





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Stakeholder Forum

group acting in concert with patients, professionals, commissioners and industry to deliver treatments that are targeted to address patients needs

Validation of the OI-QoL tool

in different Healthcare settings in Europe (supported by OIFE), North and South America and Australia which capture data on what patients wants us to focus on including currently unmeet needs

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Pediatric Endocrinology Reviews

Diabetes Nutrition Metabolism Genetics

Volume 15 Supplement 1, November 2017

The Needs of Subjects with Short Stature including Silver-Russell Syndrome and Bone Dysplasia

- ✓ *Federal association of people with short stature and their families ('Bundesverband für Kleinwüchsige Menschen und ihre Familien e.V.; BKMF)*
- ✓ *Achondroplasia (J. Spranger and B. Zabel),*
- ✓ *Silver-Russell-Syndrome (J. Salem, Netchine and M. Harbison),*
- ✓ *Pseudohypoparathyroidism (A. Linglart),*
- ✓ *X-linked Hypophosphatemic Rickets (K. Mohnike).*



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Thank you for your attention!



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Clinical trial methodologies for small populations

InSPiRe: Innovative methodology for small populations research

http://cordis.europa.eu/project/rcn/110064_en.html

IDEAL: Integrated DEsign and Analysis of small population group trials

http://cordis.europa.eu/project/rcn/109346_en.html

ASTERIX: Advances in Small Trials dEsign for Regulatory Innovation and eXcellence

http://cordis.europa.eu/project/rcn/110076_en.html

IRDiRC Small Population Clinical Trials Task Force Workshop Report and Recommendations:

http://www.irdirc.org/wp-content/uploads/2016/11/SPCT_Report.pdf



Integrated DEsign and AnaLysis
of small population group trials

