Orphan Drug Development Guidebook

Building Block E128

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	Connect 4 Children (C4C) – Paediatric Clinical Research Networks
References	https://conect4children.org/
Description	c4c (conect4children) is a large collaborative European network that aims to facilitate the development of new drugs and other therapies for the entire paediatric population. It is a pioneering opportunity to build capacity for the implementation of multinational paediatric clinical trials whilst ensuring the needs of babies, children, young people and their families are met. c4c is committed to meeting the needs of paediatric patients thanks to a novel collaboration between the academic and the private sectors, which includes 33 academic and 10 industry partners from 20 European countries, and more than 50 third parties and around 500 affiliated partners. c4c endeavors to provide a sustainable, integrated platform for the efficient and swift delivery of high quality clinical trials in children and young people across all conditions and phases of the drug development process. c4c strives to bring innovative processes to all stages of clinical development by generating a new model of organization and of the clinical development process. By emphasizing inclusiveness and collaboration across geographical, specialty, sectoral, cultural and societal backgrounds, it will set up a new infrastructure to support all

	evaluations of medicines in children. In this manner, it will become a benchmark in the currently fragmented European clinical research environment. Best practices and up-to-date expert advice will inform the c4c approaches and methods, which will subsequently be refined in the context of viability trials. The project started 1st May 2018 and has a duration of 6 years.
Category	Developmental resources Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare paediatric diseases.
Scope of use	c4c will use a coordinated approach to deliver high quality "regulatory grade" clinical trials in: – Multiple countries – Multiple sites All paediatric age groups by supporting: • Trial implementation using resources shared between studies • Trial design through a combination of information about natural history, feasibility, appropriate innovation, and expert opinion • Education and awareness within and beyond the network
Stakeholders	List of participants to the Consortium: https://conect4children.org/network/ List of Specialty Networks: https://conect4children.org/specialty-network/
Enablers / Requirements	Being part of the Consortium, applying to Call for Expression of interests.

Output	c4c promotes innovation in the design of paediatric clinical trials and quantitative methods in order to foster the development of new medicines in rare paediatric diseases and high medical needs areas. c4c aims to generate a sustainable infrastructure that optimizes the delivery of clinical trials in children through: a) a single point of contact for all sponsors, sites and investigators; b) efficient implementation of trials, adopting consistent approaches, aligned quality standards and coordination of sites at national and international level; c) collaboration with specialist and national networks; d) high quality input into study design and preparation, through rigorous strategic and operational feasibility assessment; e) the promotion of innovative trial design and quantitative scientific methods; f) an education and training platform to shape the future leaders of paediatric drug development; g) the development of sustainable support for all these activities.
Best time to apply and time window	The tool has its best use since the beginning of your product development.
Expert tips	Establish contact and follow press releases. PROs: – Structuration of the Paediatric Clinical Research Networks in Europe, public private partnership, Patient engagement within the C4C project, development of training resources CONs: Ongoing project with limited timeframe – sustainability will need to be ensured