Orphan Drug Development Guidebook

Building Block E101

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	EMA Innovation Task Force (ITF)
References	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000334.jsp&mid=WC0b01ac05800ba1d9 https://www.ema.europa.eu/en/documents/other/applying-innovation-task-force-briefing-meeting-itf-bm-step-step-guide-faq_en.pdf
Description	The ITF is a multidisciplinary EMA group that includes scientific, regulatory and legal competences. It was set up to ensure coordination across the Agency and to provide a forum for early dialogue with applicants on innovative aspects in medicines development. Area of interest: innovative therapeutics and techniques, innovative development methods, borderline products. From the first request by the applicant, the procedure lasts approximately 8-10 weeks (ITF Secretariats confirms eligibility criteria approx. within 2 weeks; when confirmed, then approx. 2 weeks about experts' involvement + meeting date after 4-6 weeks).
Category	Regulatory Building Block
Geographical scope	European Union
Availability	Applicants developing innovative methods and medicines for rare and non-rare diseases.

Scope of use	Preliminary and informally inquire all matters related to emerging therapies and technologies development with EMA, such as biomarkers (special focus on genomics) and personalized medicine, ATMPs and regenerative medicine, borderline and combined products and nanotechnology applications, especially at a very early stage of product development.
Stakeholders	• ITF secretariat (operational and scientific coordination) • ITF core members and specialized EMA staff (competences and consistency) • Experts from the EMA network (scientific expertise) • Drug developers (industry and academic)
Enablers/ Requirements	To develop highly innovative emerging therapies or technologies, in the process of entering formal procedures of the Agency. There are no enablers. This is a low threshold, informal way to initiate a dialogue with the Agency.
Output	The ITF provides an informal advice to applicants on the matter discussed at the meeting.
Best time to apply and time window	The tool may be used in early stages of development, from target selection to before human trials. In most cases, the best time is during non-clinical development before the start of any formal development activities (e.g. toxicology studies). From that stage, more formal interactions, such as Scientific Advice or Protocol Assistance, are to be used in order to get advice from the agency.
Expert tips	Especially suitable for: • applicants with highly innovative products who want to "test the water" before entering into formal interactions • applicants requiring general guidance on the next steps of development and the future use of regulatory procedures DOs: • When ITF request has been accepted, prepare **briefing** doc (max 30 pages) and **3 summary presentation slides** on: ○ description of product (e.g., structure/technology/method)

	○ Mechanism of action/Use in drug development ○ Key topics of discussion DON'Ts: • consider the ITF meeting as a substitute for a Scientific Advice/ Protocol Assistance procedure both from the perspective of meeting preparation and outcome assessment PROS: • To get help regarding all procedures • "Informal" dialogue with the Agency without generation of "binding" recommendations, which generates a "safe harbor" environment of open dialogue • Possibility to present project and/or technique also with very limited supportive data or at a very early stage of development to start a dialogue with EMA • Provides an informal feeling and flavor of the Agency's understanding of the product and of the potential issues/ red flags • Identify early the need for specialized expertise • Could facilitate dialogue with NCA at a later stage before CTA submission • Being free of charge it is accessible to any applicant, providing ITF eligibility criteria are met CONS • Participation of agency members to the meeting is less structured as other interactions, thus might not be representative of the position of the Agency • The outcome of the discussion has less weight for future regulatory interactions than a Scientific Advice/ Protocol Assistance letter • No fixed timelines • Theoretically accessible to any applicant, however eligibility criteria are defined by ITF secretariat