Orphan Drug Development Guidebook

Building Block E115

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 Small and Medium-sized Enterprises (SME) Office
References	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000059.jsp http://www.ema.europa.eu/docs/en_GB/document_library/Leaflet/2011/03/WC500104237.pdf
Description	This initiative provides support to companies assigned SME status by the EMA. The EMA's SME office promotes innovation and the development of new medicines for human and veterinary by offering financial, administrative, and regulatory assistance to micro, small and medium-sized pharmaceutical companies.
Category	Regulatory Building Block
Geographical scope	European Union
Availability	Micro, small and medium-sized pharmaceutical companies developing medicines for rare and non-rare diseases.
Scope of use	The aim is to address the increasing demand for information and support from SMEs, their stakeholders and National Competent Authorities. – Qualify as an SME enterprise (i.e., be established in the European Economic Area (EEA), employ less than 250 employees and have an annual turnover of

	not more than €50 million or an annual balance-sheet total of not more than €43 million). – Submit the form "Declaration on the qualification of an enterprise as a micro, small or medium-sized enterprise" to the EMA SME Office. – Applicants will then receive an EMA-SME number and can then benefit from the SME support.
Stakeholders	• EMA • Pharmaceutical companies (only SME)
Enablers/ Requirements	In order to benefit from the SME office, pharmaceutical companies should apply for SME status before requesting financial or administrative assistance from the Agency. To be eligible, companies must be established in the European Union (EU)/European Economic Area (EEA) and meet the definition of an SME.
Output	– direct assistance by phone, email, teleconference or through briefing meetings on regulatory aspects of the pharmaceutical legislation. SMEs receive help on how to navigate the array of services available, support in identifying the most relevant guidance, or advice on regulatory strategy for a product development or authorisation; – fee exemptions and reductions for pre- and post-authorisation regulatory procedures, including scientific advice, inspections and pharmacovigilance (see overview table below); – assistance with translations of product information into all official European Union (EU) languages for the purpose of granting an initial marketing authorisation; – inclusion in an online SME register. The register is an important source of information on EU/EEA-based SMEs involved in the manufacturing, development or marketing of medicines and promotes partnering and networking between SMEs; – guidance on clinical data publication and a free redaction tool license; – liaison with academic investigators in paediatric-medicine research through the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA); – closed workshops and training sessions held by EMA for SME only and provided for free

	– accessibility to the SME user guide (to help companies navigate through the procedures of market authorization, as well as procedures to support research and development activities)
Best time to apply and time window	This tool has its best use early during product discovery to maximise the benefits from the support offered.
Expert tips	PROs: – Provision of administrative, regulatory and financial support CONs: – You have to be a company in order to apply for SME status – If an enterprise exceeds the headcount or financial ceilings during the course of the reference year, this will not affect its situation and it will retain the SME status with which it began the accounting year. However, it will lose SME status if it goes above the ceilings for two consecutive accounting periods.