Human Medicines Division
EMA/367793/2024

Business process description

Title: Orphan status management		
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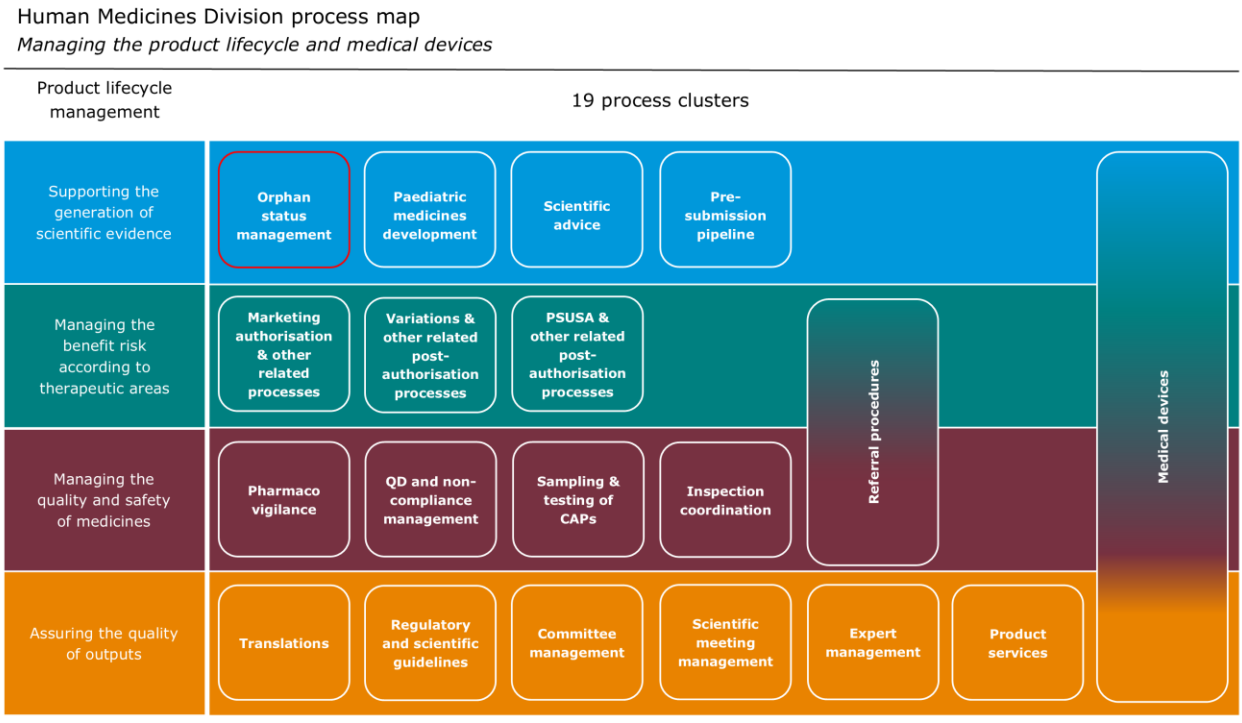
1. Introduction

The purpose of this document is to describe the high-level & end-to-end process for the orphan status management, which encourages the development and authorisation of medicines for rare diseases.

This process is part of the Human Medicines Division's process map (image below) which provides a high-level overview of the 19 process clusters within the operating model of the Human Medicines Division.



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Orphan status management process:

It describes:

- the validation and evaluation of applications for orphan medicinal product designation and amendment of an existing orphan medicinal product designation
- the review of the orphan designation at the time of an initial application for marketing authorisation of an orphan medicinal product, or an extension of indication
- the review of the market exclusivity period of orphan medicinal products

2. Changes since last revision

New business process description

3. Related documents

Procedural advice and guideline:

- Guideline on the format and content of applications for orphan medicinal product designation
- Procedural advice for orphan medicinal product designation - Guidance for sponsors
- Procedural advice for post-orphan medicinal product designation activities - Guidance for sponsors

Relevant information:

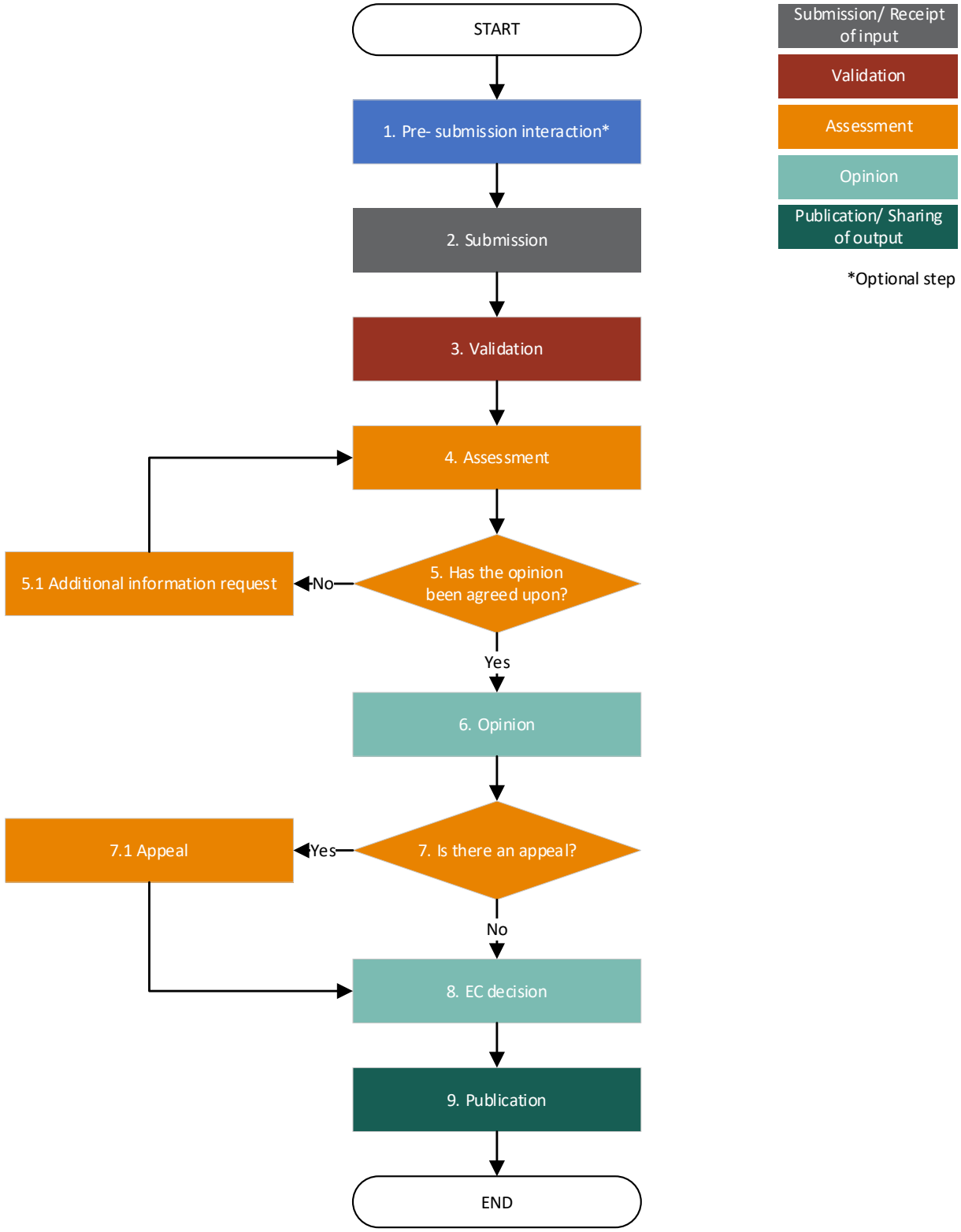
- Orphan designation: research and development
- Applying for marketing authorisation: orphan medicines
- Market exclusivity: orphan medicines

4. Abbreviations/Definitions

CAPs	Centrally authorised products
COMP	Committee for Orphan Medicinal Products
EC	European Commission
EMA	European Medicines Agency
MAH	Marketing authorisation holder
OMAR	Orphan maintenance assessment report
QD	Quality defect
PSUSA	Periodic safety update report single assessment
WOMAR	Withdrawal orphan maintenance assessment report

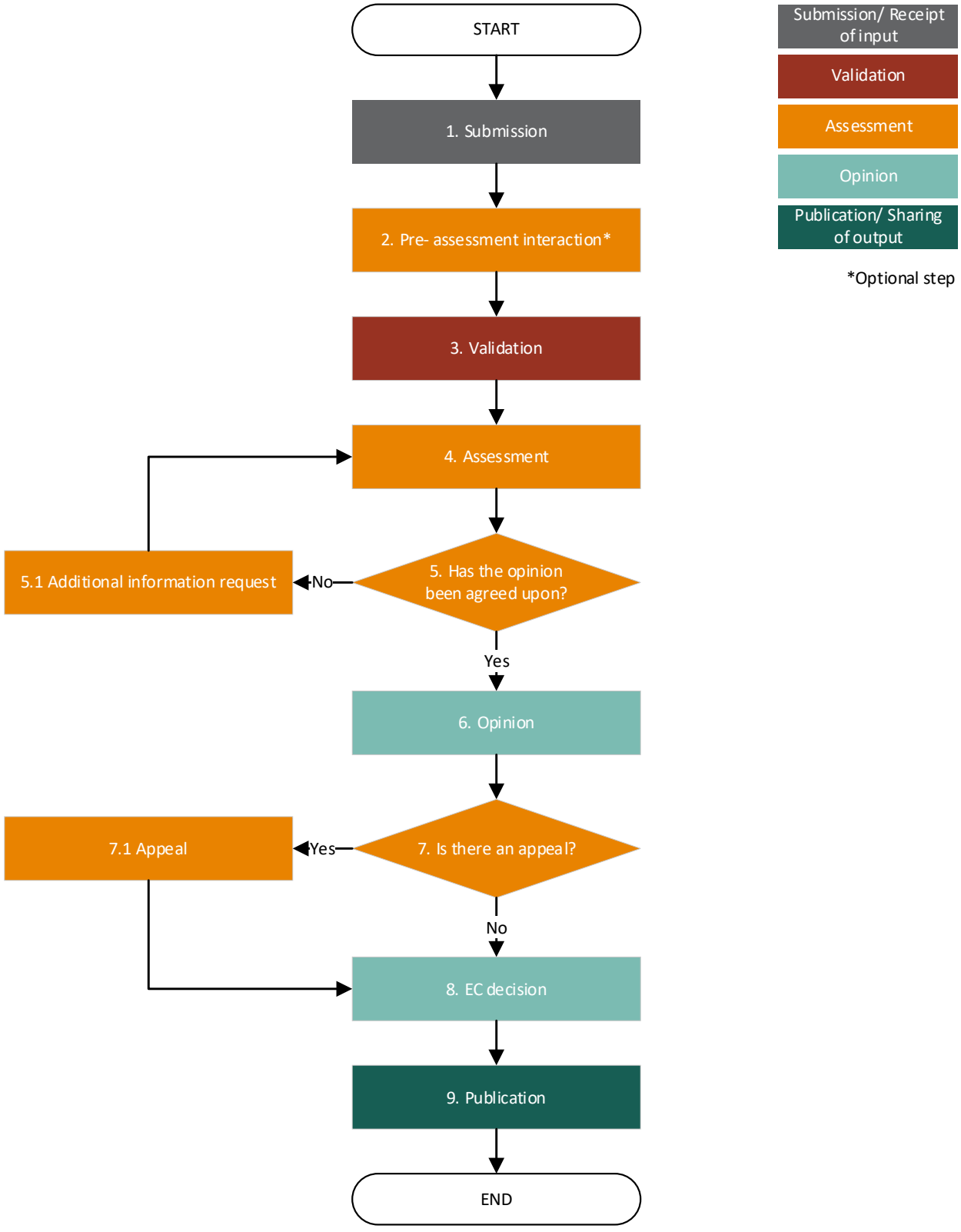
5. Process map(s)

5.1 Orphan medicinal product designation and amendment of an existing orphan medicinal product designation

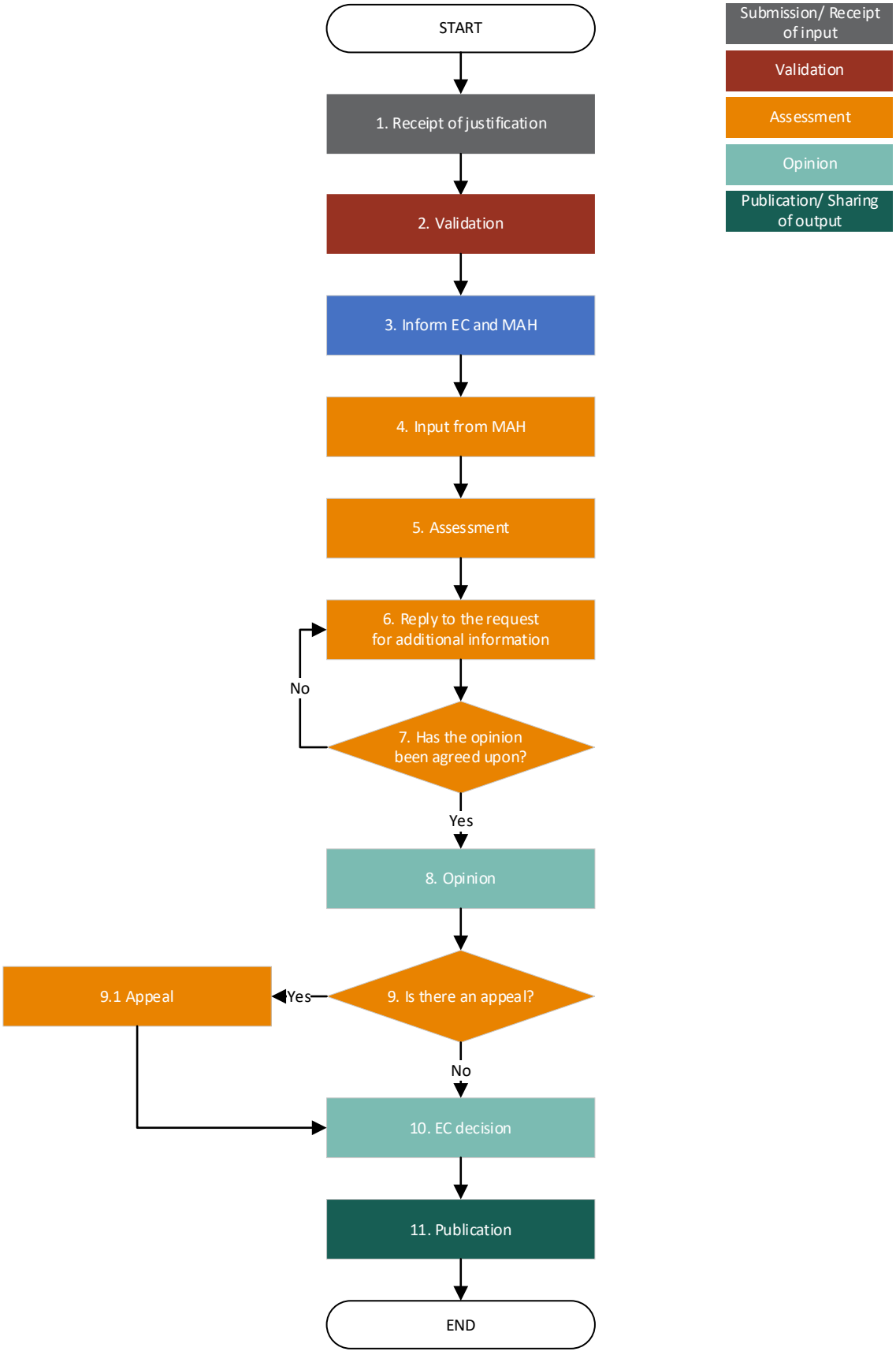


Note: Blue colour represents other steps of a process that are not covered by the above legend

5.2 Review of the orphan designation at the time of an initial application for marketing authorisation of an orphan medicinal product, or an extension of indication



5.3 Review of the market exclusivity period of orphan medicinal products



Note: Blue colour represents other steps of a process that are not covered by the above legend

6. Procedure

6.1 Orphan medicinal product designation and amendment of an existing orphan medicinal product designation

Step	Description
1.	**Pre-submission interaction** • A pre-submission meeting can be held to provide guidance to the applicant *Note: This step is optional*
2.	**Submission** • Receive submission for an application for orphan medicinal product designation
3.	**Validation** • Validate the submission *Note: Once the validation is positively concluded, the procedure starts*
4.	**Assessment** • Coordinate the assessment of the application by COMP
5.	**Has the opinion been agreed upon?** • If yes, go to step 6 • If no, go to step 5.1
5.1	**Additional information request** • Send list of questions to the applicant • A written response to the list of questions is received • An oral explanation is held, as necessary Go to step 4 *Note: This step can only occur once. The application can be withdrawn after the oral explanation.*
6.	**Opinion** • COMP adopts an opinion, and the opinion and the summary report are sent to the applicant
7.	**Is there an appeal?** • If yes, go to step 7.1 • If no, go to step 8 *Note: The appeal is only applicable when COMP adopts a negative opinion*
7.1	**Appeal** • After the appeal, COMP adopts a final opinion (go to step 8)

Step	Description
8.	**EC decision** • An EC decision is issued
9.	**Publication** • Publish the public summary of opinion

6.2 Review of the orphan designation at the time of an initial application for marketing authorisation of an orphan medicinal product, or an extension of indication

Step	Description
1.	**Submission** • Receive submission of maintenance report on the orphan drug designation criteria
2.	**Pre-assessment interaction** • A pre-assessment meeting can be held to provide guidance to the applicant *Note: This step is optional*
3.	**Validation** • Validate the submission
4.	**Assessment** • Coordinate the assessment of the application by COMP
5.	**Has the opinion been agreed upon?** • If yes, go to step 6 • If no, go to step 5.1
5.1	**Additional information request** • Send list of questions to the applicant • A written response to the list of questions is received • An oral explanation is held, as necessary Go to step 4 *Note: This step can only occur once. The application can be withdrawn after the oral explanation.*
6.	**Opinion** • COMP adopts an opinion, and the opinion and the report on the review are sent to the applicant

Step	Description
7.	**Is there an appeal?** • If yes, go to step 7.1 • If no, go to step 8 *Note: The appeal is only applicable when COMP adopts a negative opinion*
7.1	**Appeal** • After the appeal, COMP adopts a final opinion (go to step 8)
8.	**EC decision** • An EC decision is issued
9.	**Publication** • Publish the OMAR or WOMAR

6.3 Review of the market exclusivity period of orphan medicinal products

Step	Description
1.	**Receipt of justification** • Receive written justification from Member State that at least one of designation criteria of the orphan medicinal product may no longer be met
2.	**Validation** • Validate the submission *Note: Once the validation is positively concluded, the procedure starts*
3.	**Inform EC and MAH** • Provide the reasons from the Member State for triggering Art. 8(2) and the procedural timetable to the EC and MAH
4.	**Input from MAH** • Interact with the MAH • Implement their justification
5.	**Assessment** • Coordinate the assessment by COMP • COMP adopts list of questions, which are sent to the MAH
6.	**Reply to the request for additional information** • A written response to the list of questions is received • An oral explanation is held

Step	Description
7.	**Has the opinion been agreed upon?** • If yes, go to step 8 • If no, go to step 6 *Note: The COMP opinion must be adopted within the legal deadline*
8.	**Opinion** • COMP adopts an opinion, and the opinion and the assessment report are sent to the MAH
9.	**Is there an appeal?** • If yes, go to step 9.1 • If no, go to step 10
9.1	**Appeal** • After the appeal, COMP adopts a final opinion (go to step 10)
10.	**EC decision** • An EC decision is issued
11.	**Publication** • Publish the outcome of the review of the market exclusivity period