National Health Care Institute

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European network for Health Technology Assessment

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

SIGNIFICANT BENEFIT (SB) AND RELATIVE EFFECTIVENESS ASSESSMENT (REA) FOR ORPHAN MEDICINAL PRODUCTS (OMP)

Interim results

Industry stakeholder platform on research and development support

23 November 2018, EMA

Outline of study proposal

Study aim

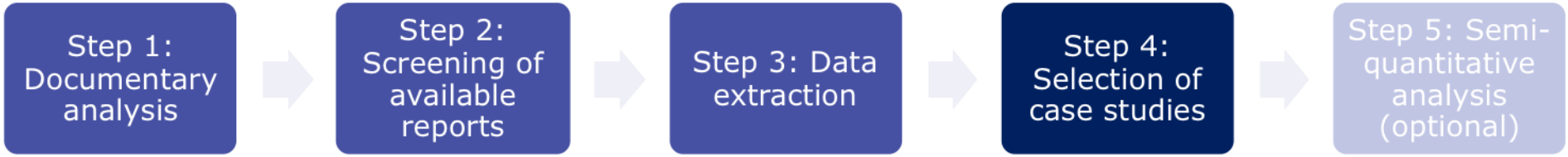
To assess the similarities and the differences between the **SB assessment** within the orphan framework assessment process as practiced by the EMA (COMP) and the **REA** as part of the HTA of orphan drugs as practiced by **HTA institutions across Europe.**

Objectives

The similarities and the differences are going to be assessed with regard to:

1. The (P)atient populations included in the assessments
2. The (I)nterventions included in the assessments
3. The (C)omparators included in the assessments
4. The (O)utcome measures included in the assessment (efficacy, safety, quality of life)
5. The role of extrapolation between patient groups
6. The use of evidence other than randomized clinical trials (RCT) used in the assessments and their role in the final outcomes of the assessments

Outline of study proposal



- **This is a qualitative, retrospective, descriptive and comparative analysis of secondary data.**
- **The output is going to be a report and a journal article containing five case studies.**

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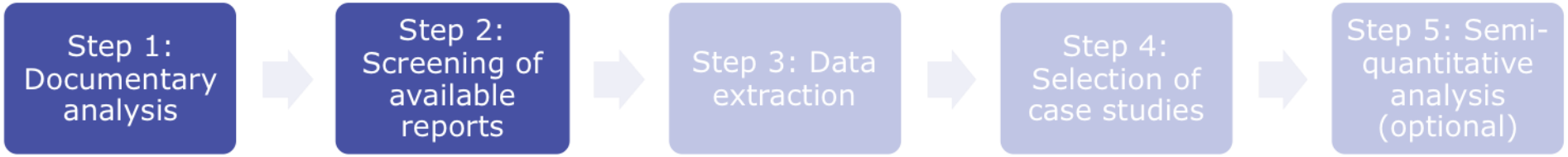

Criteria for inclusion in the study

1. MA between 2010-2017

2. Assessed for SB by the Committee of Orphan Medicinal Products at the time of MA

3. Asssessed by 4 or more of the following HTA institutions: ZIN (the Netherlands), NICE (England & Wales), HAS (France), G-BA & IQWiG (Germany) and AOTMiT (Poland).

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Progress made so far

Step 1: Documentary analysis → Step 2: Screening of available reports → Step 3: Data extraction → Step 4: Selection of case studies → Step 5: Semi-quantitative analysis (optional)

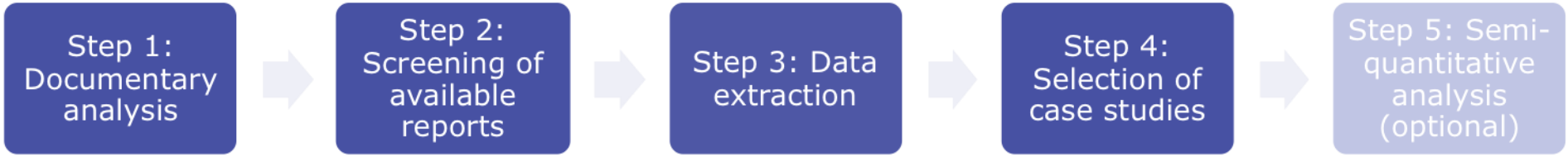
Drug-indication pairs included in the study

- 75 drugs (per indication) were found in the EMA database fulfilling the first 2 criteria
- 22 of these drugs were assessed by at least 4 HTA bodies.
- Of these 22 pairs, ZIN was involved in 13, NICE in 17, G-BA in 20, IQWiG in 4, AOTMiT in 20, HAS in 21 and EUnetHTA in 2.

The number of reports available per indication

Number of HTA reports per drug	The number of times this occurs (total 75)
6	0
5	7
4	15
3	14
2	14
1	16
0	9

Progress made so far



Case studies

1. Rydapt: EUnetHTA Rapid REA, differences in comparators.
2. Orkambi: Negative SB, different endpoints.
3. Cerdelga: Positive SB based on grounds of a ‘major contribution to patient care’.
4. Blincyto: Differences in assessments reflecting evolving evidence base over time.
5. Jakavi: Differences in comparators.

First conclusions

- SB assessment and REA frameworks share similar aspects, for 50% of cases no differences were demonstrated
- Most differences were found on the comparators considered
  - Related to methods of comparator selection (indication based vs national practices)
  - Condition vs therapeutic indication
- Detailed analysis ongoing on 5 exemplary drugs
- Based on the results and the lessons learnt from this initiative, the feasibility and the necessity of a follow-up quantitative study is going to be discussed.

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