Review of the PRIME scheme: learnings and opportunities

Presentation of high-level findings from EMA's 5 year analysis & first reflections on areas for enhancement

7th Industry stakeholder platform on research and development support, 23 November 2021

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Overview

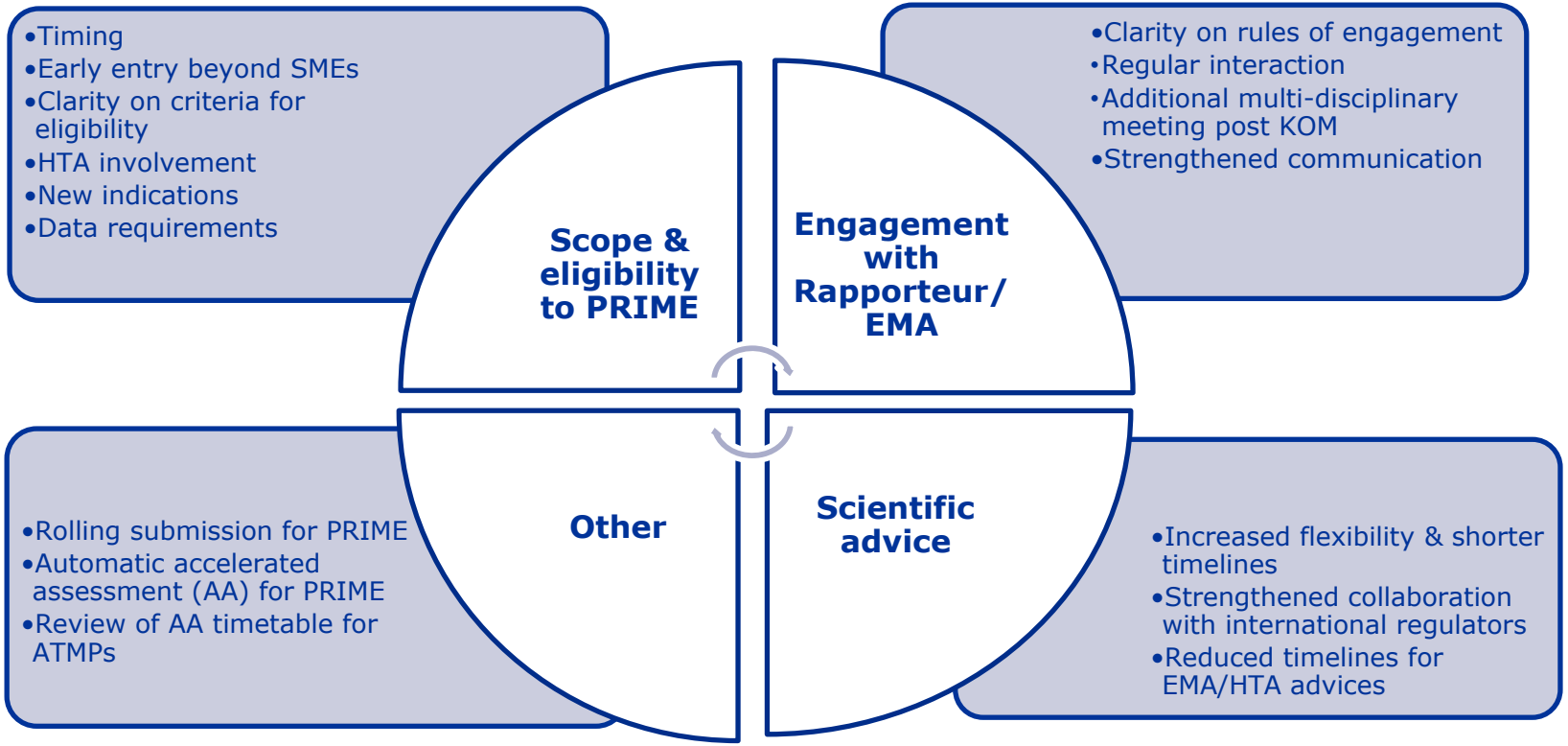
- High-level findings from 5-year analysis
  - Surveys to Companies with PRIME products
  - Surveys to Regulators
  - Analysis of PRIME applications

- First reflections on areas for enhancement of the scheme

Key findings – Surveys to Companies with PRIME products

- Overall response rate ~60%
- High level of satisfaction with PRIME support
- High likelihood to use PRIME scheme in the future
- Particular positive feedback on support offered in preparation and at kick-off meeting by EMA/Rapps. and facilitation of subsequent interactions with the Agency
- Opportunities to enhance support to product development

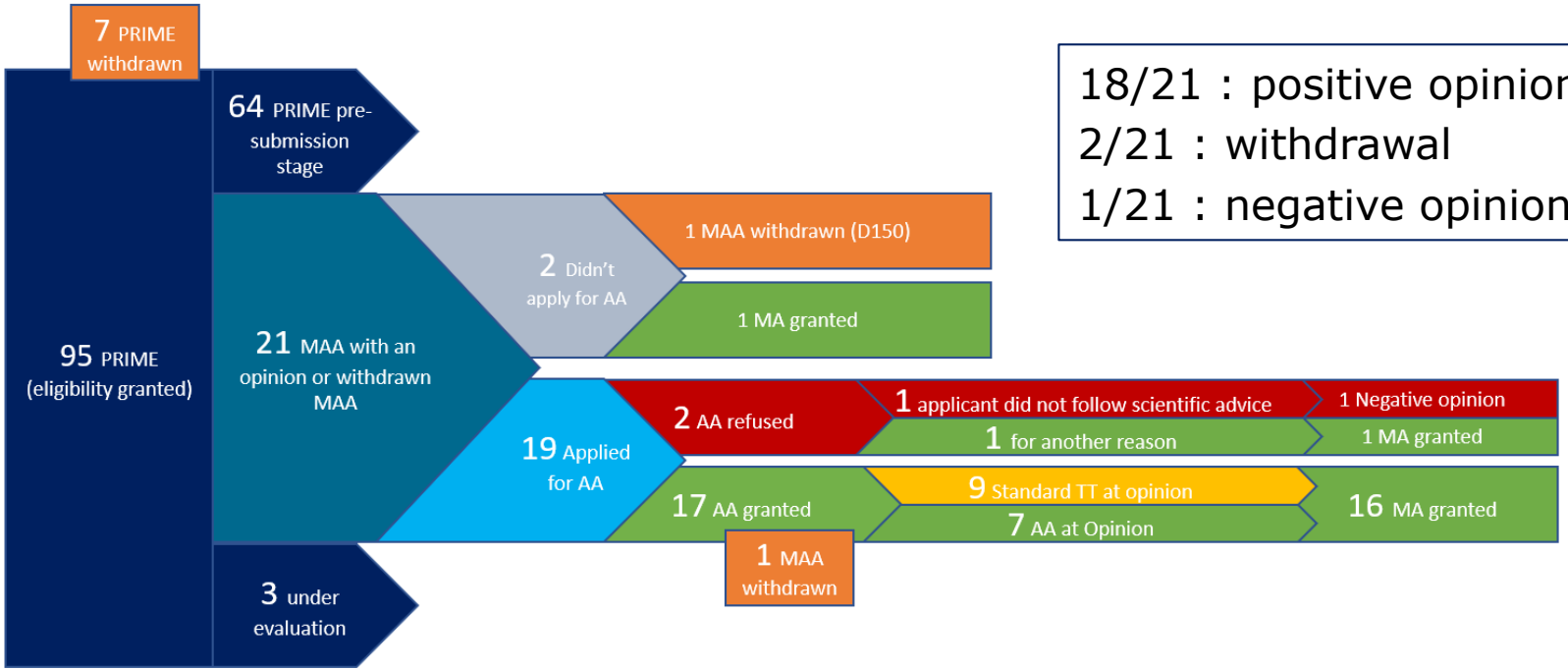
Main areas proposed by companies for improvement of PRIME



Key findings – Surveys to Regulators

- Majority considers that we are getting the right products into PRIME and at right stage of development
- Majority satisfied with current level of interaction & use of EMA to filter questions
- In terms of possible areas for enhancement of PRIME, support to strengthen knowledge building throughout development & possibility for additional interaction(s) with the Applicant at key milestones, before MAA submission

Overview of PRIME products



18/21 : positive opinion
2/21 : withdrawal
1/21 : negative opinion

Preliminary findings – **outcome of MAAs started under AA** PRIME vs non-PRIME (2018-2Q 2021)

- 17 out of 19 PRIME products started under accelerated assessment.
- PRIME products are more complex (~ 50% ATMPs, 94% orphans, higher proportion of CMAs)
- PRIME products appear more likely to **maintain accelerated assessment, despite higher complexity**.
- **Reduction of clock stop** for all category of products, most significantly for SME applicants.
- The only SME product and the only ATMP which maintained accelerated assessment were PRIME.
- Unclear relevance of observed difference in the number of major objections (MOs).

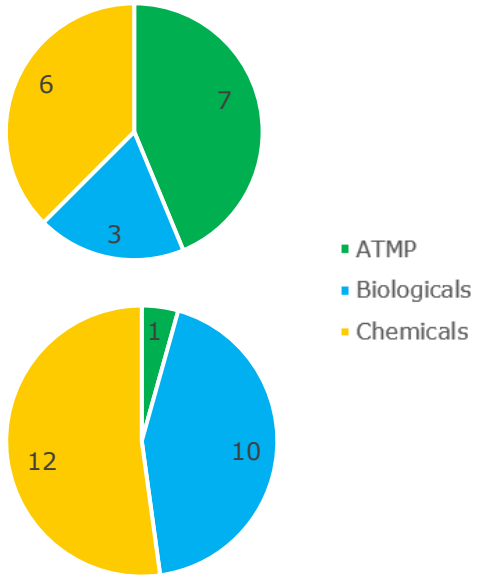
Methodology

- All data presented in these slides refers to medicinal products which started evaluation under accelerated assessment (AA) and had a final opinion from the CHMP between 01 January 2018 and 30 June 2021
- Sample of 16 PRIME and 23 non-PRIME

Category of products started under accelerated assessment

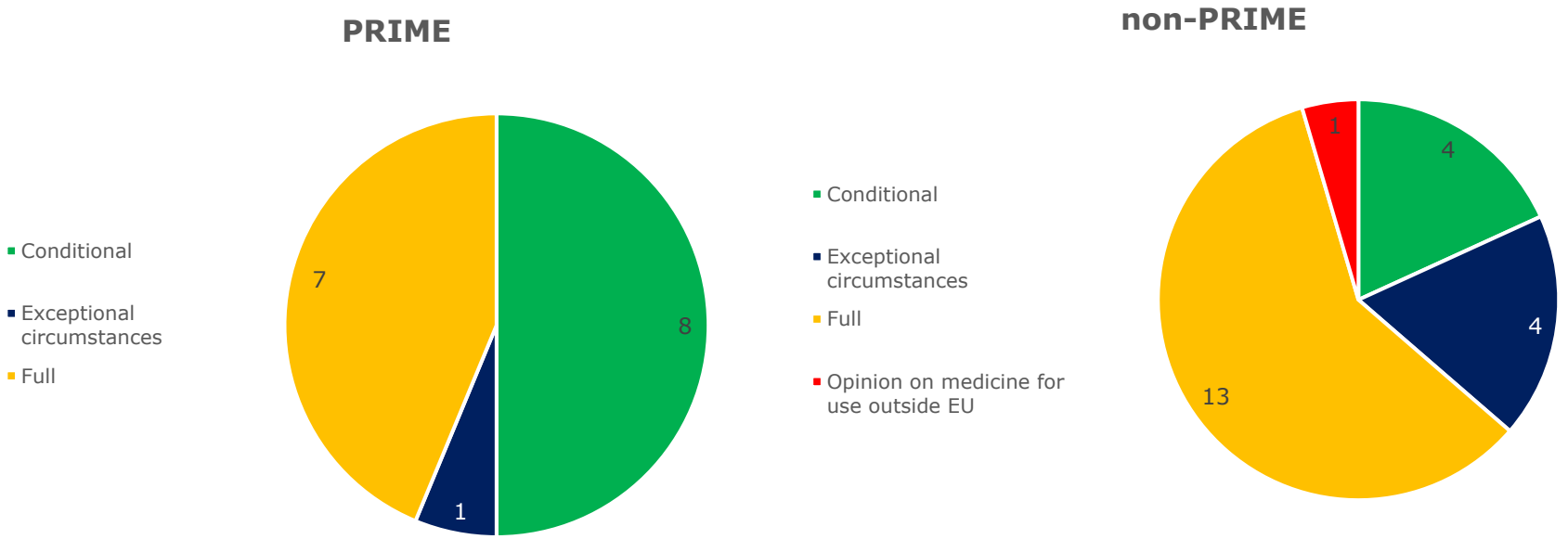
PRIME

non-PRIME

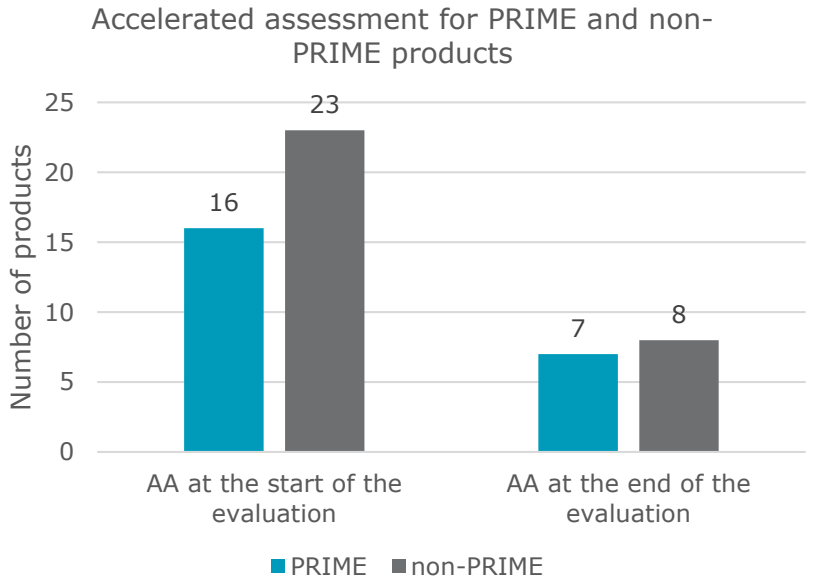


- **44%** of PRIME products are **ATMP** and only 4% of non-PRIME products.
- Amongst the products which started under accelerated assessment, the PRIME products are more complex than the non-PRIME products

Type of marketing authorisation for product started under AA

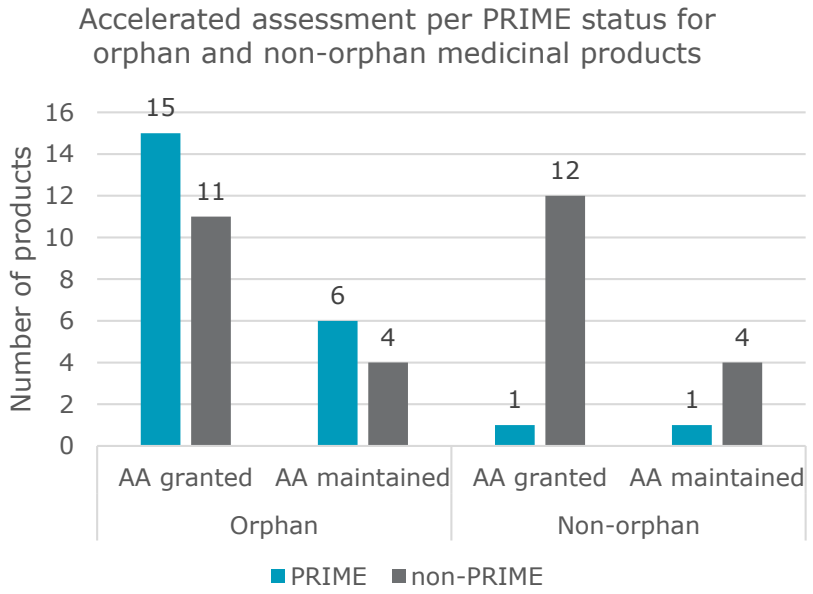


Maintenance of Accelerated Assessment



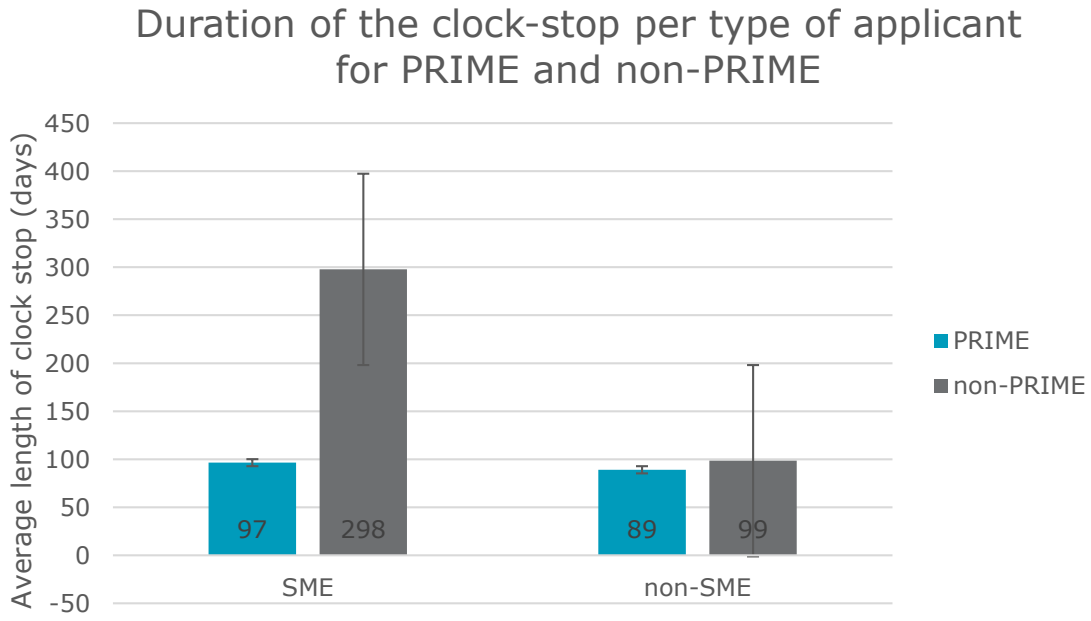
- **44%** of PRIME products keep accelerated assessment until the end of the evaluation stage (7/16).
- **35%** of non-PRIME products maintain it (8/23).
- PRIME products appeared less likely to switch to a standard timetable, despite increased complexity.

Maintenance of accelerated assessment for orphan medicinal products



- **94%** of PRIME medicinal products under accelerated assessment were designated as orphan and only 48% of non-PRIME under accelerated assessment.
- **58%** of the orphan medicinal products which are granted accelerated assessment are PRIME (15/26) and **60%** of those which maintain accelerated assessment (6/10).

Duration of clock stop per applicant type



- For **SMEs**, there is a **201-day** decrease of the clock stop for PRIME products compared to non-PRIME products.
- For non-SME, there is a 10-day reduction.

What have we learnt?

Findings show that PRIME helps MA assessment, particularly for the more difficult products (ATMP, SME)

The first 5y of PRIME ran in a challenging resource environment.

These challenges have shown that:

- We need to maximise effectiveness of approach.
- We need to cut the dead branches (activities of limited value) and adjust what is in the right direction for PRIME to be a meaningful program

What are we trying to achieve?

Support the development of deserving products in areas of UMN.

Design actions based not on a fixed process, but focused on effective support and oversight.

Facilitate the work of Rapporteurs in the run up to MAA, including knowledge building and access to up-to-date information.

Eliminate automatic interactions and documents of limited utility (annual update).

Create an agile process that makes it easier to follow the development and whether the product still is on track to fulfil its 'promise'

Areas to explore for a benefit/effort proportionate process

Flexibility of SA provision for PRIME

- Support continuity of assessment teams and EMA (incl. GXP)
- Agile and flexible provisions (formal and informal)
- Response proportionate to real urgency
- Develop guidance for different level of interaction

Knowledge building to support AA

- Tracking development progress rather than calendar dates
- Meetings time and content should relate to utility
- Facilitate Rapporteur knowledge building
- Leveraging IRIS for knowledge building to support tracking issues during development

PRIME eligibility: scope and timing

- **What do we want PRIME to be?**
- Maximise impact on shaping development: consider best timing
- Involve the best expertise available, at the right time.
- Support access and global development- Parallel SA: HTA and FDA

Next steps and timelines

- Planning Report publication Q1 2022
- Follow-up discussion with industry stakeholders to support implementation of actions to further enhance the value of the PRIME scheme