# 23: Correlation between publication of pivotal clinical data and marketing authorisation: a case study of orphan drug approvals from 2021–2023

## Background

- Effective publication planning plays a pivotal role in the adoption of a new drug by ensuring that primary safety and efficacy data are available to enable healthcare professionals to make informed prescribing decisions

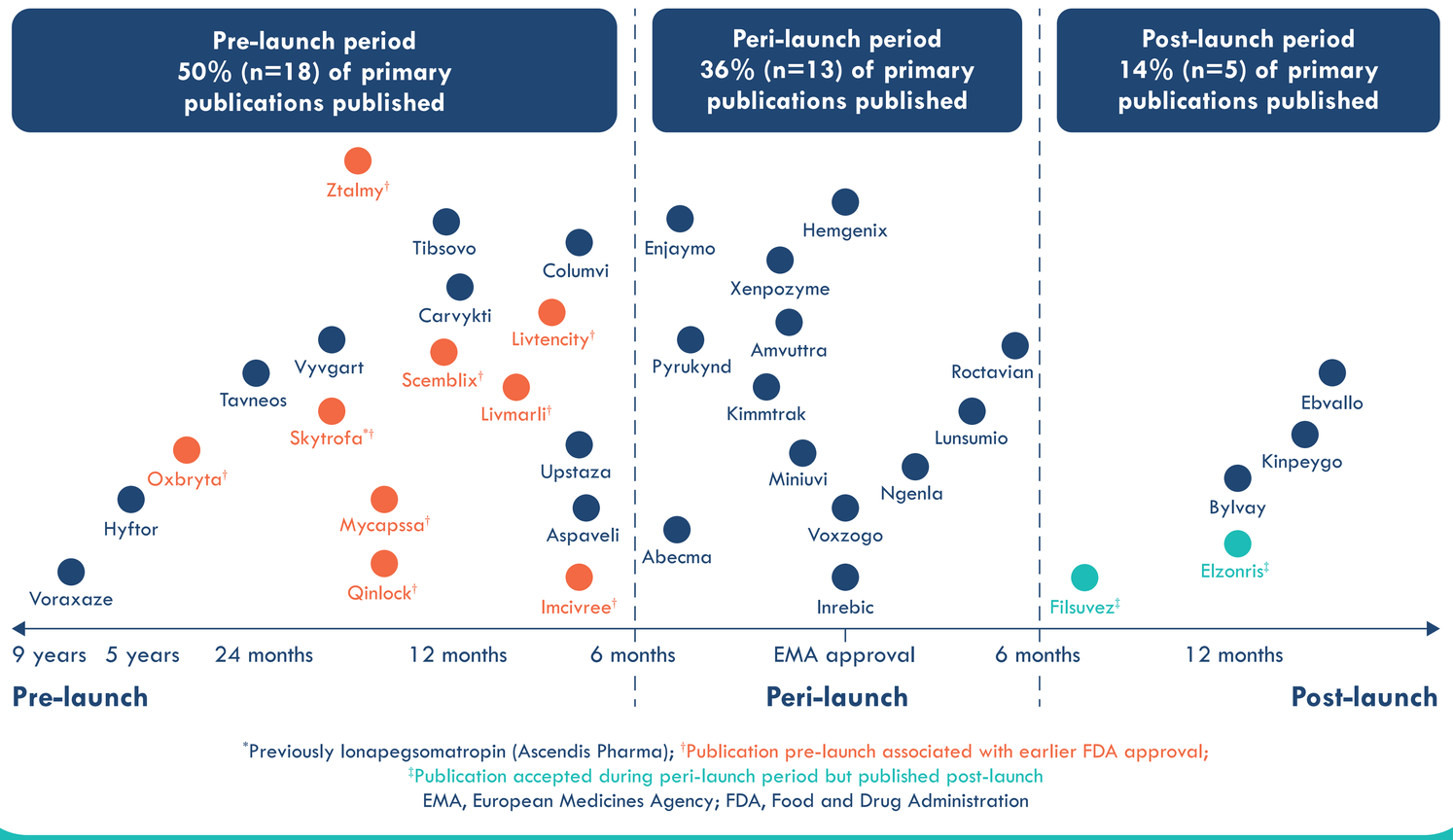
## Aims

- We assessed the timing of primary data publication in relation to approval of new orphan drugs by the European Medicines Agency (EMA) from 2021–2023 using an automated approach

## Results

- Primary publications could not be identified for three of the 39 orphan drugs approved from 2021–2023. Of the remaining 36:
  - 13 (36%) were published peri-approval
  - Of the 18 (50%) published >6 months before EMA approval, nine were published within 6 months of an earlier US Food and Drug Administration (FDA) approval
  - Two of the five publication (14%) published >6 months after approval were accepted for publication within 6 months of EMA approval
- No association between either number of authors or number/proportion of industry authors and the timing of primary publication was identified

Figure 1: Timings of primary publication of data for 36 orphan drugs mapped to pre-, peri- and post-launch periods for EMA approval




*Previously lonapegsomatropin (Ascendis Pharma); †Publication pre-launch associated with earlier FDA approval; ‡Publication accepted during peri-launch period but published post-launch
EMA, European Medicines Agency; FDA, Food and Drug Administration

## Conclusions

- Publication of pivotal clinical trial data outside of the peri-launch period was common, emphasising the need for effective publication planning
  - Publication >6 months pre-approval can provide opportunities for competitors to counter-message. It may also reduce the impact of data as healthcare professionals are unable to prescribe the relevant drug when the data are published
  - Publication post-approval can delay uptake of new drugs due to healthcare professionals not having access to peer-reviewed data to support use upon approval

CLINICAL DATA

Primary data for **36%** of orphan drug approvals were published within the peri-launch period

IMPLICATIONS

OPPORTUNITIES

## Future considerations

- This analysis considered orphan drug approvals only, the process for which may differ to that for regular drug approvals. Whether a similar pattern of data publication is observed for regular drug approvals or in different therapy areas needs to be evaluated
- Failure to provide clinical trial identifiers in Summaries of Product Characteristics (SmPCs) and differences in data between SmPCs and primary publications make it challenging to match publications to approved drugs. Inclusion of clinical trial identifiers in SmPCs should be mandated to support full interpretation of clinical data for approved drugs

## Methods

- New orphan drug approvals between July 2021 and July 2023 were identified; approvals of secondary indications and of biosimilar products were excluded
- Data collated for each approved drug included:
  - Date of regulatory submission
  - Date of approval
  - Key clinical trial(s)
  - Date of the primary publication of pivotal clinical data (PubMed)
- The timing of primary publications was classified as pre-launch (>6 months before EMA approval), peri-launch (within 6 months before or after EMA approval) or post-launch (>6 months following EMA approval)

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