

ORPHAN DRUG DESIGNATION: NO IMPACT ON P&R PROCESS TIMING IN ITALY

Andrea Enrici, Dario Lidonnici, Martina Isernia, Elena Paola Lanati

MA Provider, Milan, Italy

Background

The marketing authorization of orphan drugs (drugs indicated for the diagnosis, prophylaxis or treatment of life threatening and/or debilitating rare diseases) is a responsibility of the European Medicines Agency (EMA). The Committee for Orphan Medicinal Products (COMP), in particular, has the task to establish whether a drug can be designated as an orphan.

To qualify for orphan designation, a medicine must meet a number of criteria (life threatening or chronically debilitating disease, prevalence less than 5 in 10.000 and no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition).

Thanks to important economic and access incentives recognised to Pharma Companies for the development of orphan drugs, the number of applications is significantly increasing over time.

Objective

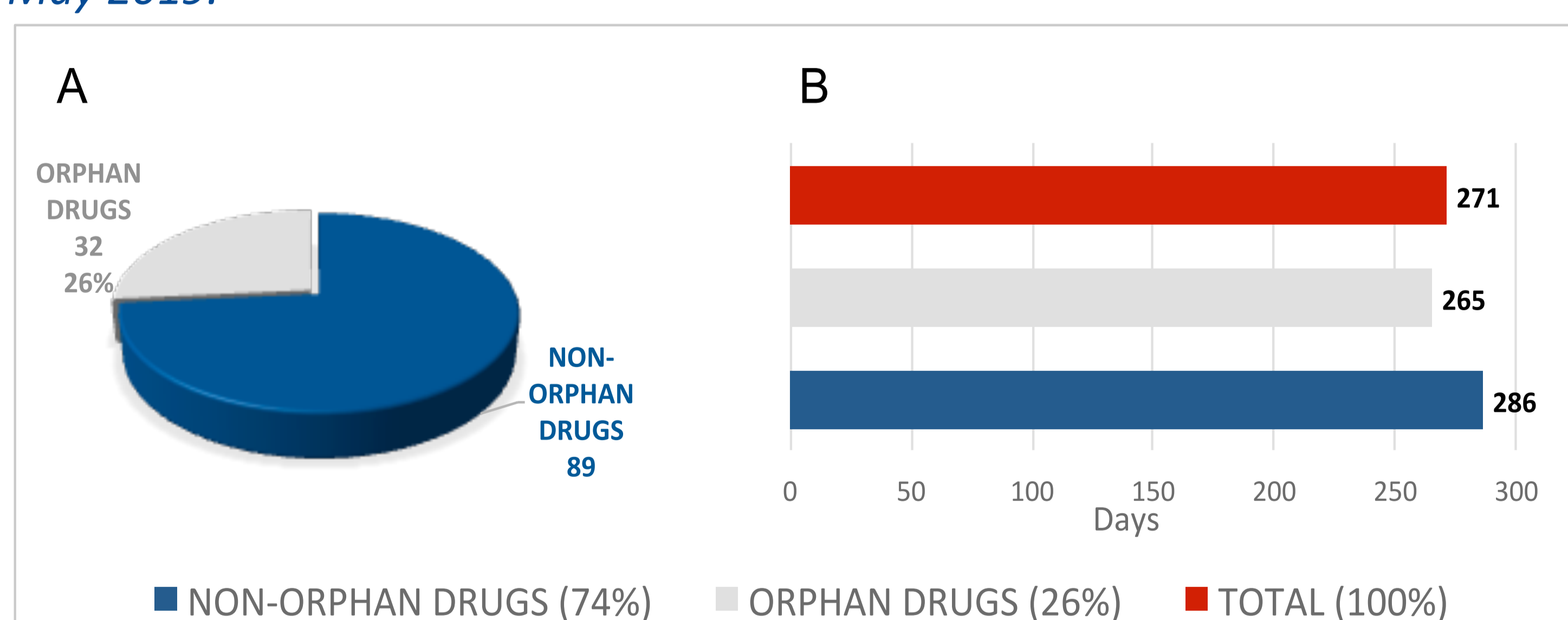
The main purpose of this analysis was to quantify the time to market (TTM) of innovative drugs and analyse the differences between orphan vs non-orphan drugs to understand if the innovative status recognition has an influence on the P&R process.

Methods

All medicines of product reimbursed in the period between January 2015 and May 2019 were tracked: were analysed in terms of timing according to different categorizations (non-innovative vs innovative status; with vs w/o MEA; oncological vs non oncological indications).

The analysis was conducted starting from EMA website, monthly reports of AIFA committees meeting, Official Gazettes and orphan designated drugs.

Figure 1. Sample considered for the analysis (A) and average approval timing for orphan vs non-orphan drugs reimbursed in Italy between January 2015 and May 2019.



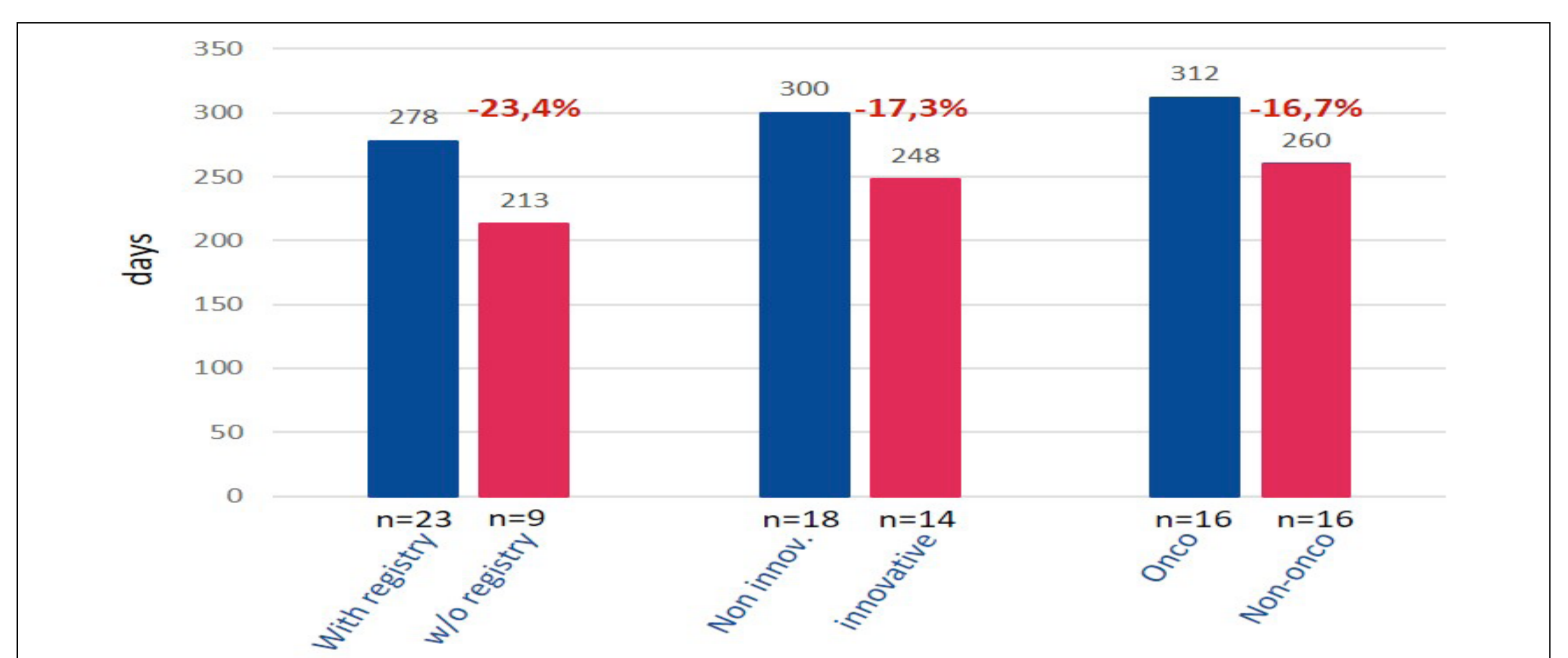
Results

The analysis of the approval timing regarding all reimbursed drugs (N=121; 100%) from January 2015 till May 2019 resulted in 271 days on average from CTS opening to Official Gazette publication.

The sample included 32 orphan drugs (26%) and 89 non-orphan drugs (74%) (Figure 1A) and results in terms of time to reimbursement were similar between the two categories: 265 vs 286 days respectively (Figure 1B).

Categorization by therapeutic area yielded a 312 days P&R process for oncological orphan drugs (n=16, 13%) compared to 260 days for non-oncological orphan drugs (n=16, 13%). In case AIFA granted innovative status to an orphan drug (n=14, 12%), the process took 248 days on average. The presence of a monitoring registry also impacts on the approval timing: 23 orphan drugs (19%) with monitoring registry have a longer process compared to 9 (7%) orphan drugs without registry (278 vs 213 days) (Table 1). The P&R process of orphan drugs is mostly impacted by committees assessment timing, while post assessment (bureaucratic) phase remained comparable (about 87 days).

Figure 2. Timing and number of orphan drugs stratified by presence of a monitoring registry, innovative status recognition and therapeutic area (oncological vs non oncological indications).



Conclusions

The analysis showed a similar TTM for orphan drugs compared to non-orphan drugs: despite unmet medical need is well recognised in rare diseases, further research by AIFA (due to limited data availability and knowledge on the specific disease) may be needed, thus extending assessment timing.

Therapeutic area has a significant impact on timing of P&R process, probably due to the need of even more detailed assessment for oncological orphan drugs compared to non-oncological orphan drugs. The combination of orphan designation and innovative status accelerates the approval process, highlighting the key role recognised by AIFA to innovation, which translate into a faster time to reimbursement.

References

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