

# NEGOTIATION OUTCOMES IN ITALY: WHAT'S THE IMPACT OF ORPHAN DRUG DESIGNATION?

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## 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.

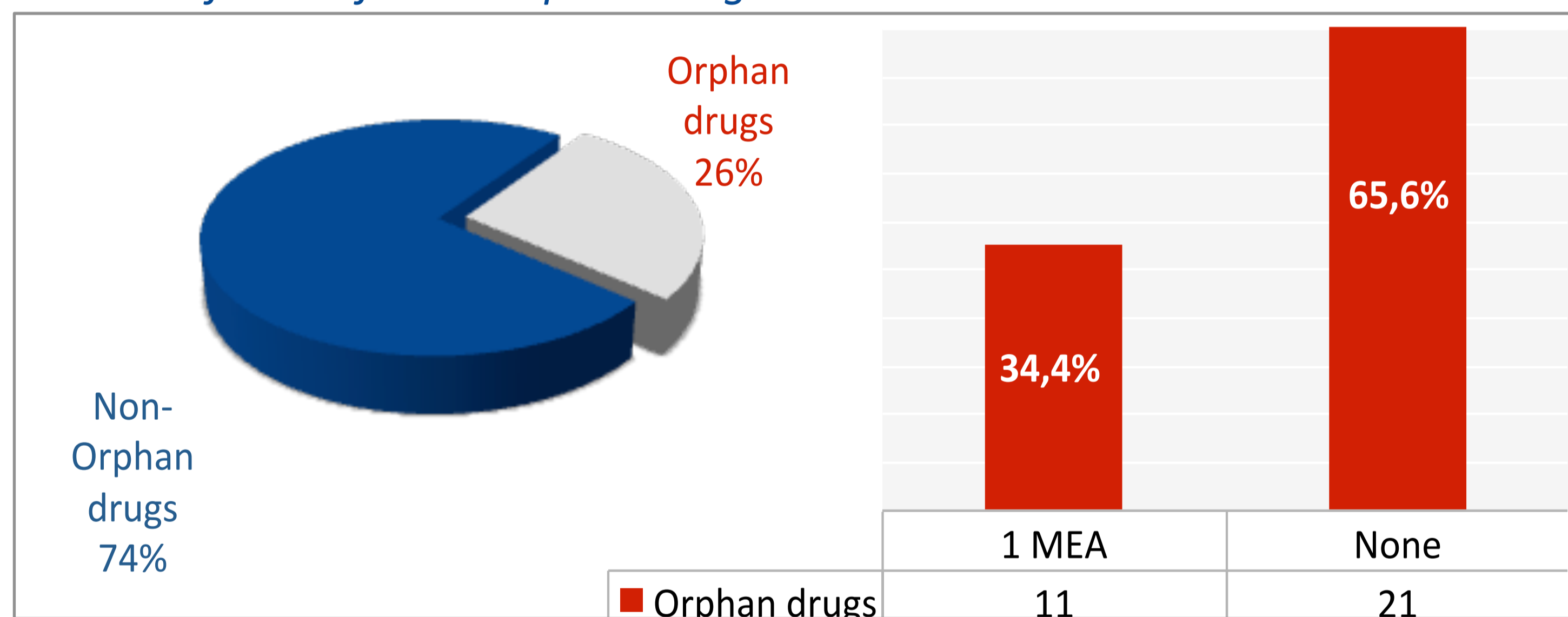
Managed Entry Agreements (MEAs), monitored by AIFA through the use of specific Monitoring Registries, have been introduced in June 2005. Italy is recognised as one of the pioneers in developing access schemes for new medicines.

MEAs between AIFA and Pharma Companies could be "financial/non-outcome-based" or "performance/outcome-based".

## Objective

The objective of the present work is to analyse the negotiated conditions (Managed Entry Agreement, discount and monitoring registry) of drugs designated orphan by EMA and reimbursed in Italy from January 2015 to May 2019, to understand possible correlations, which could be helpful for Pharma Companies in terms of strategy implemented action and outcome expectations.

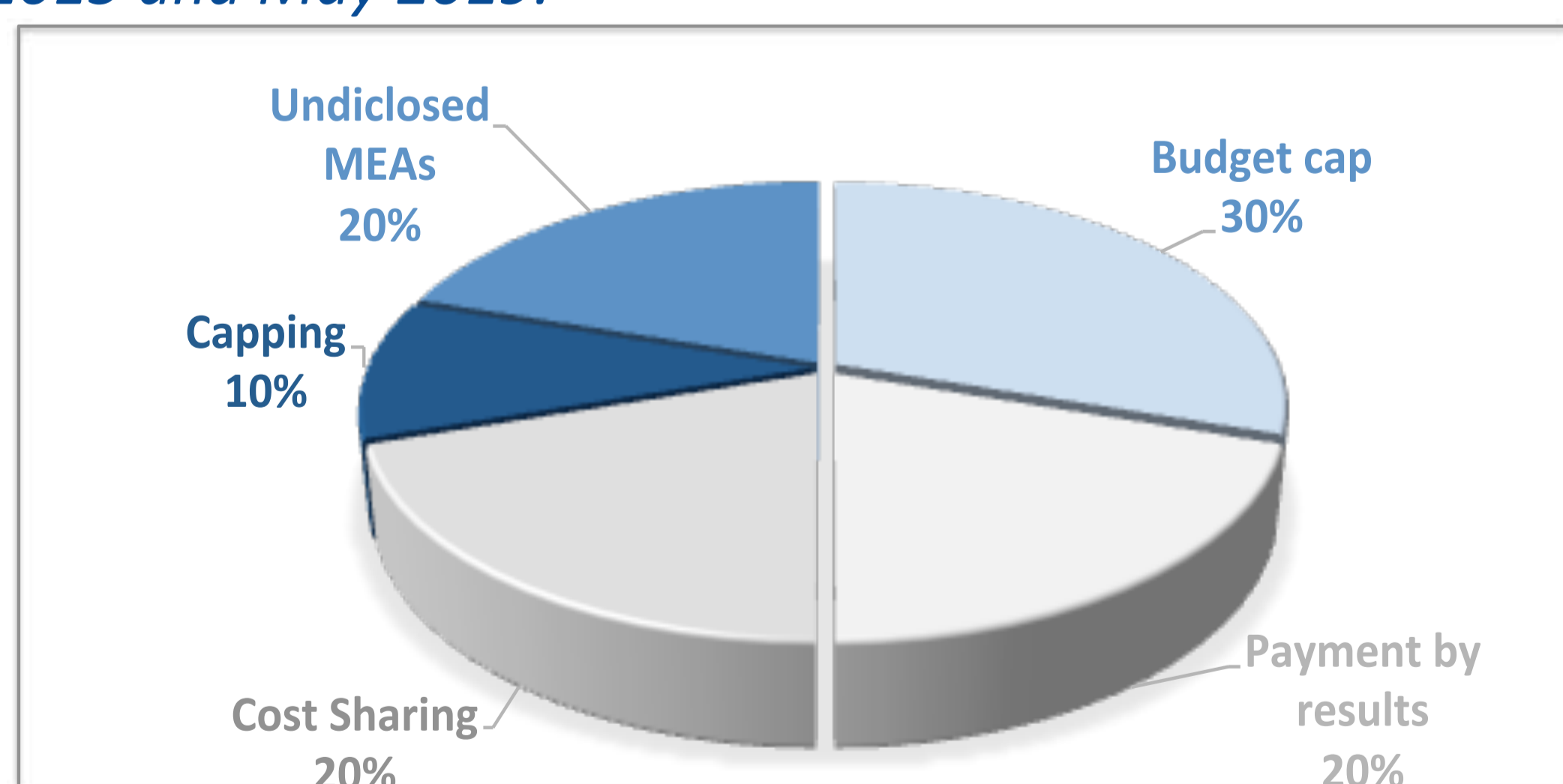
**Figure 1.** Reimbursed drugs by AIFA: non-orphan drugs vs orphan drugs and number of MEAs for the orphan drugs.



## Methods

The analysis was conducted starting from monthly reports from AIFA committees meeting, EMA website and AIFA orphan drug lists. P&R process of newly reimbursed orphan drugs was tracked in internal database and a sub-analysis by therapeutic area was also run. Published Official Gazettes were used to source the presence of monitoring registries, confidential hidden discounts and MEAs as negotiation outcomes.

**Figure 2.** MEAs applied by AIFA for all orphan drugs reimbursed between January 2015 and May 2019.



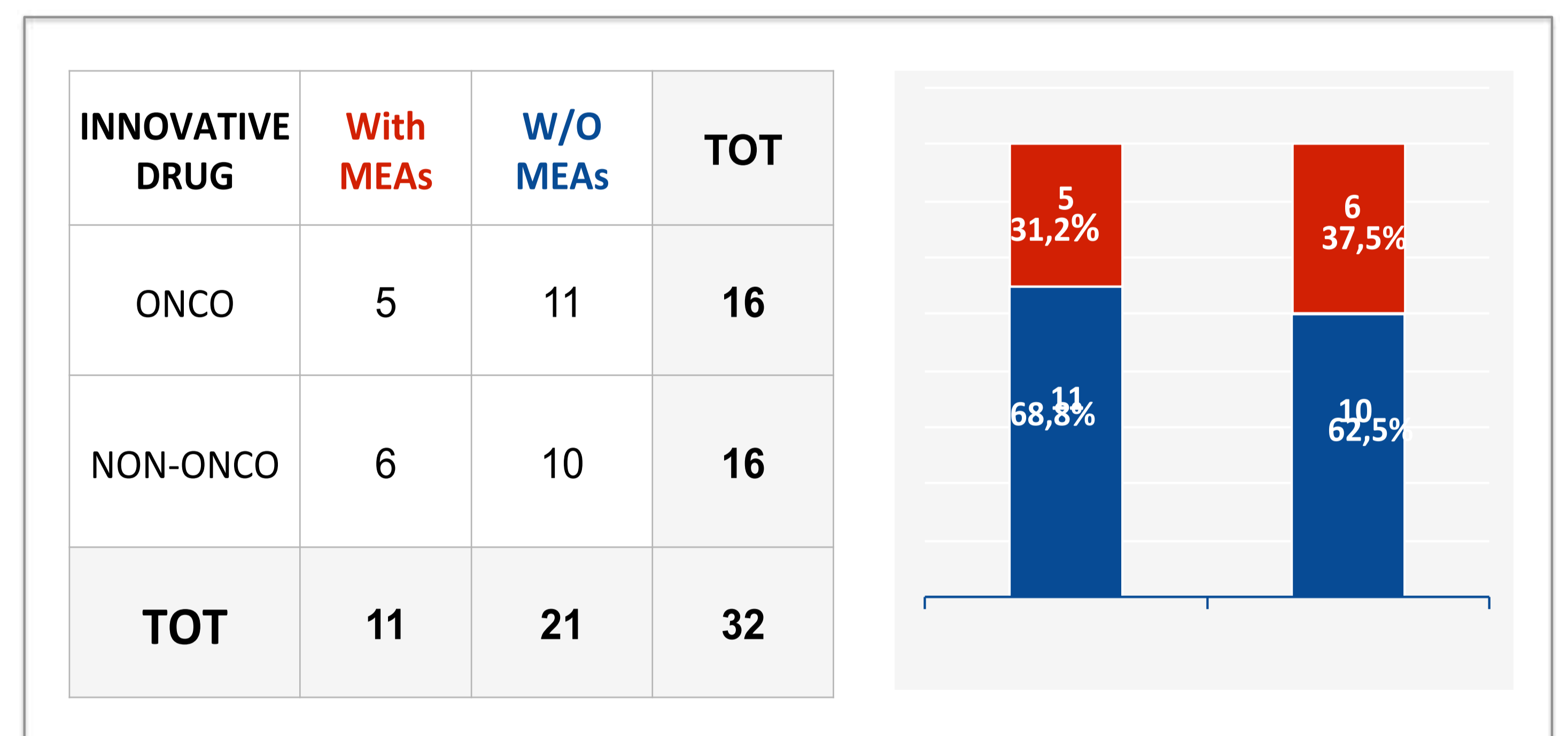
## References

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- [http://www.bollettinosifo.it/r.php?v=2598&a=26748&l=329644&f=allegati/02598\\_2016\\_06/fulltext/07\\_Ex%20Lege.pdf](http://www.bollettinosifo.it/r.php?v=2598&a=26748&l=329644&f=allegati/02598_2016_06/fulltext/07_Ex%20Lege.pdf)
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## Results

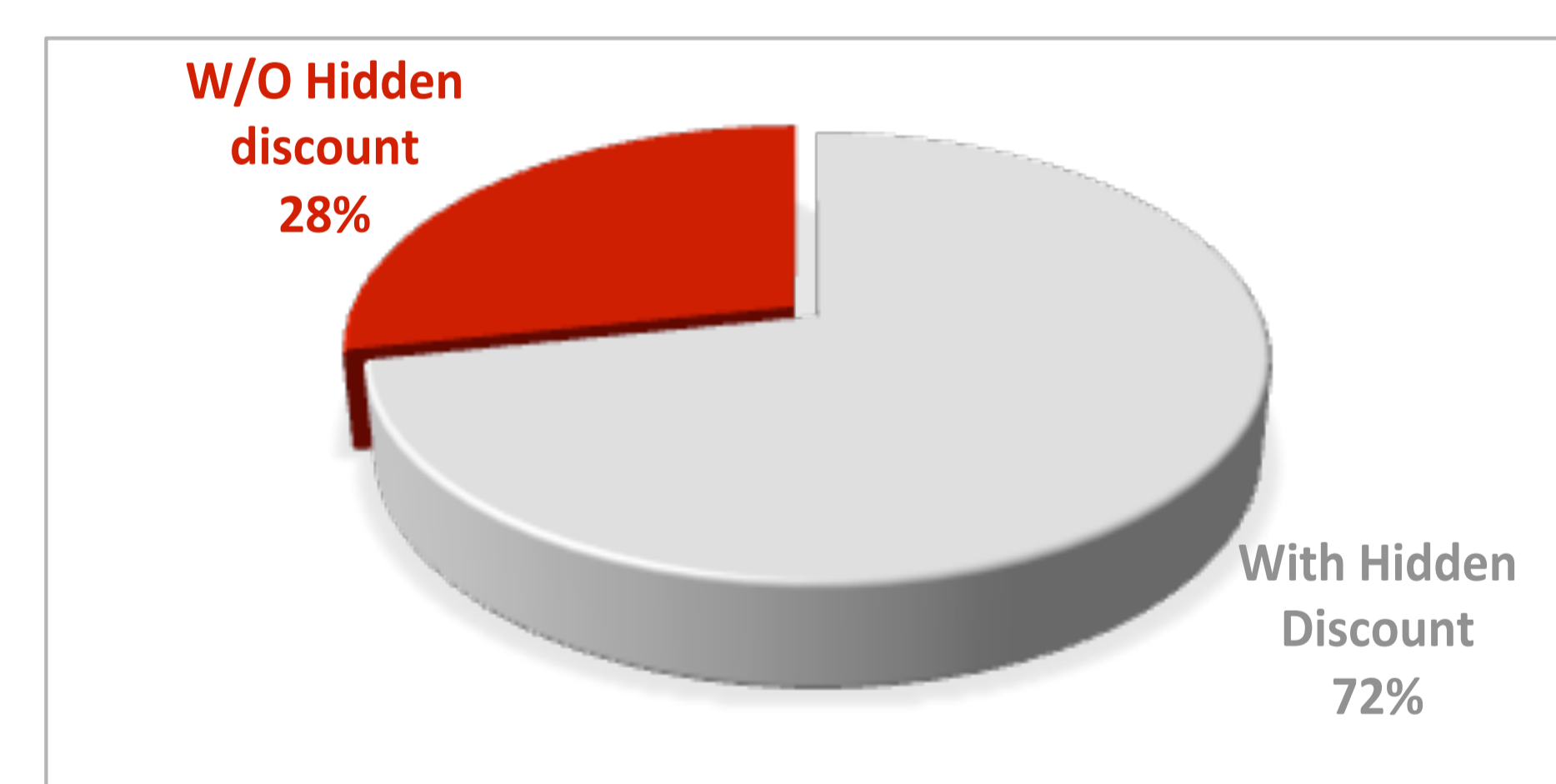
During the analysed period 32 out of 121 (26%) reimbursed drugs were also orphan according to EMA designation (Figure 1). The results regarding the application of MEAs to the orphan drugs show 21 out of 32 (66%) had no MEAs and 11 out of 32 (34%) had one single MEA as approval condition. Focusing on the 11 MEAs applied, we tracked: 3 (9.4%) budget caps, 2 (6.3%) cost sharing agreements, 2 (6.3%) payment by results, 1 (3.1%) capping, 2 (6.3%) undisclosed MEAs (Figure 2). Categorization by therapeutic area yielded 5/16 (31.3%) oncological orphan drugs with MEAs and 6/16 (37.5%) non oncological orphan drugs with MEAs (Figure 3).

**Figure 3.** Oncological orphan vs non-oncological orphan drugs with and w/o MEAs.



Additionally a flat fee per patient was applied to 1 drug (3.1%). 23 out of 32 (71.9%) orphan drugs had a monitoring registry, and 23 (71.9%) had confidential discounts (Figure 4).

**Figure 4.** Hidden/Confidential discount applied by AIFA for all orphan drugs reimbursed from January 2015 to May 2019.



## Conclusions

The analysis showed a majority of orphan drugs without MEAs. Therapeutic area did not impact on MEAs assignment, given the similar percentage of oncological orphan vs non oncological orphan drugs with MEAs. There is a preponderance of financial MEAs compared to outcome-based MEAs. Although orphan drugs are a peculiar category of medicinal products, often conditionally approved or under exceptional circumstances or with lack of solid evidences, Italian authority keeps focusing on budget management rather than possible clinical outcomes uncertainties, maybe also due to difficulties in measuring performances and thus in applying outcome-based MEAs.