

≥95% Discounts!?

Is Canada a Commercially Viable Market for Orphan Medicines?

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INTRODUCTION

- Canada's Drugs Agency (CDA formerly CADTH), is the HTA body for most of Canada (except Quebec). CDA provides expert advice to provincial payers and the pCPA (Canada's pan-provincial price negotiator) on the clinical- and cost-effectiveness of medicines
- CDA publishes publicly-available reports that typically include clinical restrictions where necessary, and the percentage discount on the list price required to achieve sufficient cost-effectiveness
- Orphan drugs can face greater access challenges than other medicines due to their typically less robust clinical evidence packages combined with their higher prices
- This research reviewed CDA/CADTH HTA assessments of orphan drugs published over the last 7 years, focussing on the magnitude of discount CDA/CADTH judged necessary to be deemed cost-effective in Canada

METHODS

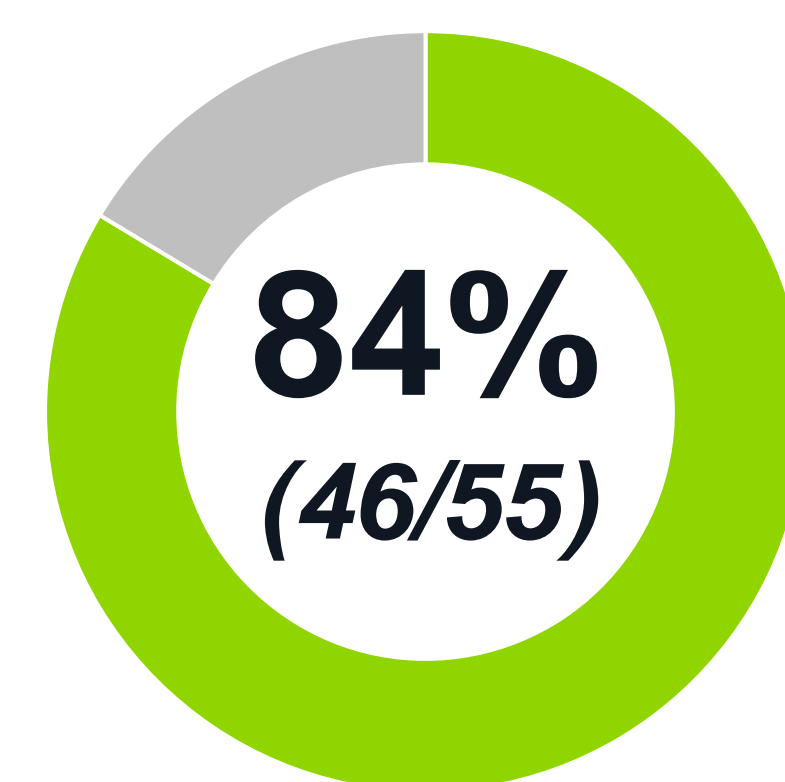
- A list of orphan drugs launched between 2017 and 2023 (inclusive) were identified using EMA data (as Health Canada does not provide a list of the drugs which have been granted orphan status)
- For each of these products, key data were extracted from the associated CDA/CADTH appraisals, including overall recommendation, any restrictions, list price, recommended percentage-discount, and the resulting recommended net price
- Where key data were not published, the entry was excluded; where uncertainty led to CDA/CADTH providing a recommended discount in the form of a range, a crude mean of these percentages were used in our analysis

RESULTS

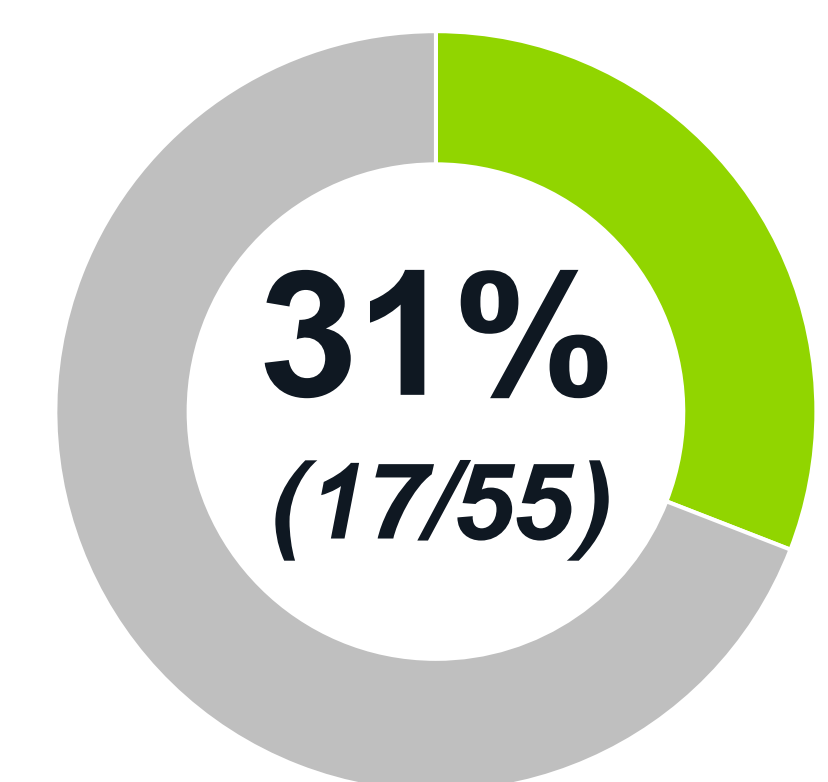
Using the methods described, 55 orphan therapies were identified for analysis. Of these 55 orphan therapies:

CDA/CADTH median recommended discount was:

85%



Discount >50%



Discount ≥95%

Distribution of discounts recommended by CADTH by product type

- Oncology orphan medicines fared slightly better (median discount: 76%, IQR: 56%-87%) than orphan drugs overall
- Conversely, therapies for ultra-orphan conditions were associated with higher discounts (median discount: 92%, IQR: 85%-95%)
- Five CAR-T cell therapies were included, which had a median recommended discount of 80% (IQR: 76%-89%)
- The two gene therapies, Luxturna and Zolgensma, had recommended discounts of 74% and 95%-99% respectively

Drug grouping	n	Discount %			
		Mean	1 st quartile	2 nd quartile	3 rd quartile
All drugs	55	73%	60%	85%	95%
Oncology	19	71%	56%	76%	87%
Ultra-orphan	16	84%	85%	92%	95%
CAR-Ts	5	78%	76%	80%	89%



CONCLUSIONS

- Our analysis provides a valuable insight into health-economic value perception for new medicines in Canada, as well as a bellwether for other CEA markets, highlighting stringent net price pressure
- It is worth noting that the magnitude of the recommended discounts may partially reflect arbitrarily high list prices
- Additionally, Canadian payers are not bound to CADTH recommendations and may accept a less substantial discount at the point of negotiation and reimbursement

