POSTER #106

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Do prices and doses differ for initial and follow-on indications? An analysis of multi-indication orphan drugs

Ching-Hsuan Lin, MD, MPH[1]; Molly T. Beinfeld, MPH[1]; Claire Brennan, BA[1]; Julia A. Rucker, MSW, MPH[1]; Tom Hughes, PhD[2]; Glenn Phillips[2], PhD; James D. Chambers, PhD[1]
1. Tufts Medical Center, Boston, MA; 2. Argenx, Inc., Boston, MA, USA

CEVR
Center for the Evaluation of Value and Risk in Health

BACKGROUND

- A drug's unit price (e.g., per milligram) is fixed under US uniform unit-based pricing.
- If a follow-on indication requires higher doses, the annual treatment costs are typically higher, even if the clinical value doesn't.
- Example: Maralixibat costs $578K/yr for Alagille syndrome (initial indication) vs. $1.32M/yr for PFIC (follow-on).

OBJECTIVE

To characterize price and dose differences for multi-indication orphan drugs

METHODS

- We calculated price and dose ratios for follow-on indications vs. first indications.
- **Sample:** Drugs with multiple orphan indications approved by FDA (2000 -2024)
- We characterized reasons for dose differences:
  - Dosing metric (e.g., body weight- vs. body surface area-based dosing)
  - Treatment duration (continuous vs. fixed-duration treatment, such as a 12-week course)
  - Dose magnitude (higher daily doses such as 10 mg vs. 5 mg)
  - Age group (adult vs. pediatric)

Data sources:

- Doses: FDA labeling + clinical trial data
- Prices: List price from Red Book

RESULTS

565 novel orphan drugs (2000-2024) → 105 had multiple orphan indications → 43 had varying doses and pricing data

- The final sample included 43 drugs and 70 follow-on indications.
- **56%** (39 of 70) of follow-on indications **required higher doses.**
- **79%** (31 of 39) of those had **higher annual treatment prices.**

Table 1. Dose and price ratios for follow-on indication vs. first indication (n = 70)

Ratio	Mean (SD)	Median (IQR)
Dose	4.4 (20.5)	1.3 (0.7-2.0)
Price	2.4 (3.9)	1.0 (1.0-2.0)

Table 2. Reasons for dose differences among follow-on indications

Reason	Dose ratio			
	< 0.5	0.5 ≤ and < 1	< 1 and ≤ 2	> 2
Dosing metric	3	2	1	2
Treatment duration	5	1	3	4
Dose magnitude	3	8	13	10
Age group	1	3	1	5

Note: Categories are not mutually exclusive.

- The most frequent reason for dose differences was **dose magnitude** (n=34), followed by **treatment duration** (n=13).

CONCLUSION

Follow-on indications for multi-indication orphan drugs **often require higher annual doses** and, under uniform pricing, a **higher annual price.**

IMPLICATIONS

- Because unit-based pricing ties price to dose rather than to clinical value:
  - Higher-dose indications are more costly and therefore may face more stringent payer coverage restrictions.
  - Lower-dose indications (such as pediatric) yield lower revenues per patient, potentially reducing development incentives.
- Aligning price with clinical value rather than dose could better align incentives.

LIMITATIONS

- We relied on list rather than net prices.
- Mean dosing assumptions may not reflect patient-level variation.
- Focus on orphan indications limits generalizability.

CONTACT & QR code

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