Alignment of Health Plan Reauthorization Criteria with Clinical Trial Endpoints for Neuromuscular Disease Therapies

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BACKGROUND & OBJECTIVE

- Neuromuscular diseases (NMDs) are progressive conditions that impact nerve and muscle function, often leading to immobility and requiring long-term treatment.[1]
- Many health plans have specific criteria that patients must meet to qualify for continuation of treatment. Prior research shows that health plans often impose coverage restrictions and reauthorization criteria for NMD therapies.[2]
- Our study examined the alignment between health plans' reauthorization criteria for NMD therapies and the endpoints included in the therapies' registration studies (collected from ClinicalTrials.gov).

METHODS

Data Source

- We used coverage information as of December 2024 from the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) Database, which tracks specialty drug coverage decisions for 18 NMD therapies from 18 large US commercial health plans.

Analyses

- We analyzed reauthorization criteria and categorized criteria into:

1. **General/adequate response:** *e.g., evidence of a positive treatment response.*
2. **Require specific clinical outcomes:** *e.g., a symptom score below a defined threshold on a disease-specific scale.*

- For decisions requiring specific clinical outcomes, we compared health plan requirements with registration clinical trial endpoints, and categorized alignment as:

1. **Identical:** *Identical to clinical trial endpoint (same measure, same threshold).*
2. **Partially Consistent:** *Same measure but different threshold, or some additional criteria.*
3. **Additional Criteria Only:** *Included only measures not assessed in clinical trials.*

RESULTS

- We identified 29 NMD drug-indication pairs in our sample. SPEC included 401 coverage decisions, 303 (75.6%) included reauthorization criteria. Health plans ranged from 34.6%-92.0% of coverage decisions including reauthorization criteria.
- Of these 303 policies:
  - 74 (24.4%) required a general/adequate response.
  - 229 (75.6%) required specific clinical outcomes.
- Among policies requiring specific clinical outcomes:
  - 66 (28.8%) were identical to clinical trial endpoints, 140 (61.1%) were partially consistent, and 23 (10.0%) only included additional criteria (Figure 1).
- Across the 18 health plans:
  - The share of reauthorization criteria identical to clinical trial endpoints ranged from 0% to 61.5%; partially consistent ranged from 28.6% to 82.3%; and additional criteria ranged from 0% to 28.6% (Figure 2).

Figure 1. Reauthorization Coverage Decision Breakdown

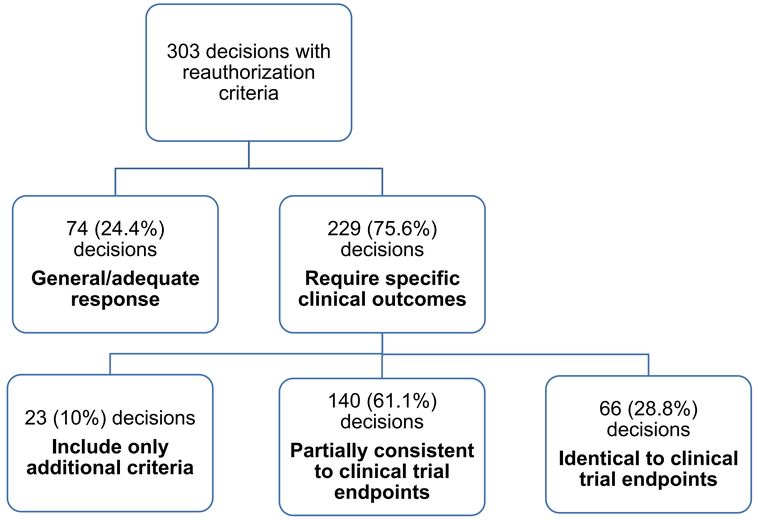
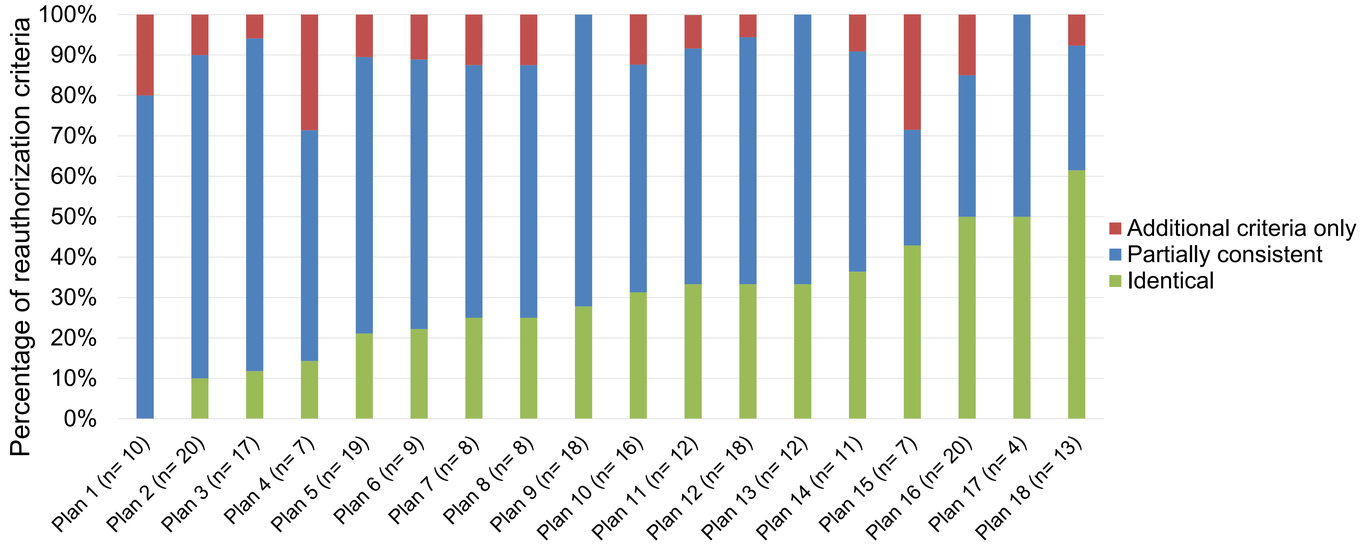


Figure 2. Variation in Alignment Between Reauthorization Criteria and Clinical Trial Endpoints Across Health Plans



CONCLUSION

- Reauthorization criteria for NMD therapies vary widely across health plans and plans frequently diverge from clinical trial endpoints.
- While most plans require specific clinical outcomes for continued coverage, plans often use clinical outcome measures beyond those studied in clinical trials.
- Even when the same measures are used, plans may apply more restrictive thresholds than in trials.
- These findings suggest a disconnect between health plan reauthorization criteria and evidence-based clinical practices. These findings highlight the need for greater consistency and transparency in coverage reauthorization decisions.
- Future research should examine how health plans determine reauthorization criteria and why these criteria may diverge from clinical trial evidence.

REFERENCES

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CONTACT

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