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Does Accelerated Approval Result in Favorable Access: Examining the Burden of Evidence Necessary for Appropriate Payer Coverage of Accelerated Approval Products

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OBJECTIVES

The FDA accelerated approval (AA) pathway allows patients early access to drugs treating serious conditions with high unmet need. Accelerated approval products have historically received varying payer coverage, ranging from no coverage to coverage in line with the labeled indication. This analysis aims to understand the key factors influencing payer coverage of accelerated approval products.

METHODS

Non-oncology, non-infectious disease accelerated approval products launched from 2014-2023 with ongoing confirmatory trials were isolated for analysis. Isolated products were reviewed to understand the extent of clinical evidence supporting approval. Key factors analyzed included trial design (e.g., Advisory Committee (Ad Comm) votes, endpoint selection, objectivity and functionality of endpoints), disease prevalence, number of current competitors, magnitude of effect, safety & tolerability, and annual cost. Endpoints were assessed for **objectiveness** (e.g., measured without personal bias, such as lab results, physical measurements, etc.) and **functionality** (e.g., direct impact on patient symptoms, such as walking distance, reductions in lesions, etc.).

Subsequently, coverage was assessed across a representative sample of 10 commercial plans for all therapies meeting the research criteria and grouped into 3 categories: coverage in line with the labeled indication, coverage with restrictions beyond the labeled indication, or no coverage. Current wholesale acquisition cost (WAC) prices were also calculated for each product to understand expected economic impact. Lastly, we analyzed all collected information to understand the influence of supporting clinical evidence and price on the commercial payer coverage landscape.

Products Analyzed in Research

Product	Manufacturer	Approval Date	Objective Endpoint	Functional Endpoint
Ocaliva	Intercept Pharmaceuticals	5/27/2016	Y	Y
Vonjo	CTI Biopharma	2/28/2022	Y	Y
Galafold	Amicus Therapeutics	8/10/2018	Y	N
Vijoice	Novartis Pharmaceuticals	4/5/2022	Y	Y
Voxzogo	Biomarin Pharmaceutical	11/19/2021	Y	Y
Northera	Lundbeck	2/18/2014	N	N/A
Skysona	Bluebird Bio	9/16/2022	Y	Y
Filspari	Travere Therapeutics	2/17/2023	Y	N
Andexxa	Astrazeneca	5/3/2018	Y	N
Oxbryta	Global Blood Therapeutics	11/25/2019	Y	N
Qalsody	Biogen	4/25/2023	N	N/A
Amondys 45	Sarepta Therapeutics	2/25/2021	Y	N
Exondys 51	Sarepta Therapeutics	9/19/2016	Y	N
Viltepso	Nippon Shinyaku	8/12/2020	Y	N
Vyondys 53	Sarepta Therapeutics	12/12/2019	Y	N
Elevidys	Sarepta Therapeutics	6/22/2023	Y	N

Table 1. Summary of Products Included in Research
In the case where a product failed its primary endpoint, endpoints used for AA were analyzed

RESULTS

After an analysis of the 16 pharmaceutical products meeting our research criteria across a broad range of critical factors, data suggest products with endpoints grounded in objectivity and functionality during pivotal clinical trials receive more favorable payer coverage.

Of the 8 products with the most favorable payer coverage, 5 products used objective and functional endpoints in their pivotal trials. One such product, Skysona, is likely managed more restrictively due to its high cost as a gene therapy. Galafold has relatively favorable coverage for a product without a functional primary endpoint, although the biomarker studied (GL3) has been linked by existing products to improved clinical outcomes. Additionally, 5 accelerated approval products had Ad Comm meetings; products with strong advisory committee support receive more favorable coverage by payers. Analyzed results may be skewed by DMD products, which all receive more restrictive payer coverage.

Current Coverage Status of AA Drugs

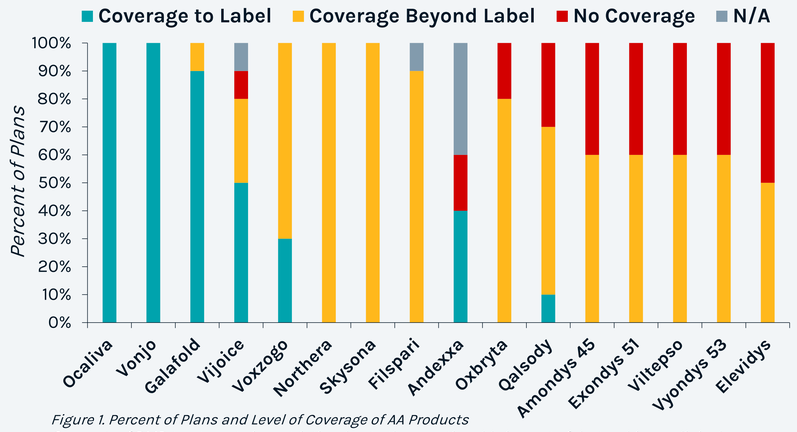

Figure 1. Percent of Plans and Level of Coverage of AA Products
Coverage of DMD treatments is restrictive due to the questionable impact of dystrophin on clinical outcomes; Aduhelm was not included due to its recent discontinuation, although likely would have the most restrictive coverage of all products

Ad Comm Votes

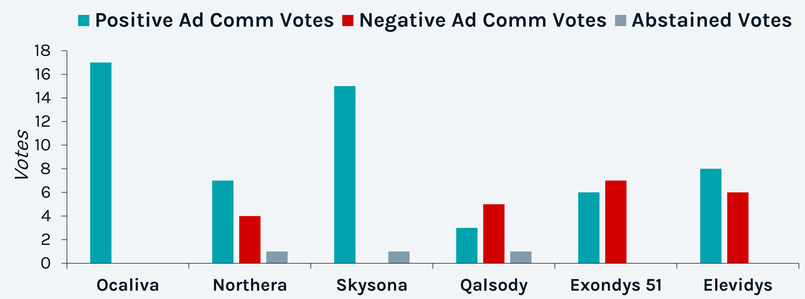

Figure 2. Advisory Committee Breakdown of Votes for AA Products

CONCLUSIONS

The primary factor driving payer coverage appears to be the functionality of the primary endpoint. Additionally, the presence of fewer competitors at launch, a greater efficacy magnitude, and a perceived alignment of value and annual cost of treatment are likely to also influence coverage. Unlike subjective assessments or biomarker-based endpoints, these objective, functional endpoints are demonstrably linked to improved outcomes in two key domains:

1. **Payer Coverage:** Objective, functional endpoints seem to facilitate less restrictive coverage. This trend implies a stronger acceptance of these products' value and efficacy from the payer's perspective, leading to favorable coverage. A detailed review of reimbursement patterns shows that insurers are more inclined to cover treatments that present clear, measurable benefits, as seen in reduced hospitalizations and lower long-term healthcare costs.

2. **Ad Comm Endorsement:** Analysis also reveals a propensity for Ad Comm meetings to cast more favorable votes for products with objective and functional endpoints. This pattern indicates an enhanced confidence in the product's clinical significance, safety, and effectiveness from the scientific and medical community. Deliberations from recent Ad-Comm meetings highlight the committees' preference for robust, quantifiable data that can be reliably replicated, enhancing the credibility of the research findings.

FUTURE IMPLICATIONS

Given that AA products are awaiting confirmatory trial results, the evidence is inherently incomplete, leading to payer skepticism about product value. This skepticism may cause a misalignment between the price-value relationship of AA products and payer expectations, potentially affecting coverage decisions. To secure favorable access, manufacturers must optimize evidence generation strategies by using objective/functional measures that address payer concerns about product performance. This can help mitigate disparities in the price-value relationship, especially as payers increasingly prioritize cost management.

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