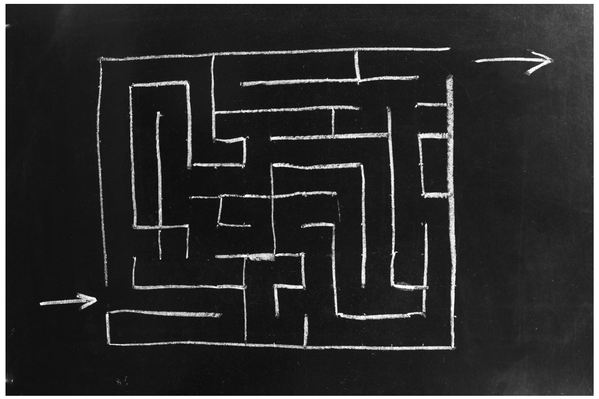
What is ‘fair’ access for orphan drugs in the U.S.?

Monday, 08 May 2023 | 1:45 PM - 2:45 PM ET
Boston, Massachusetts

Welcome

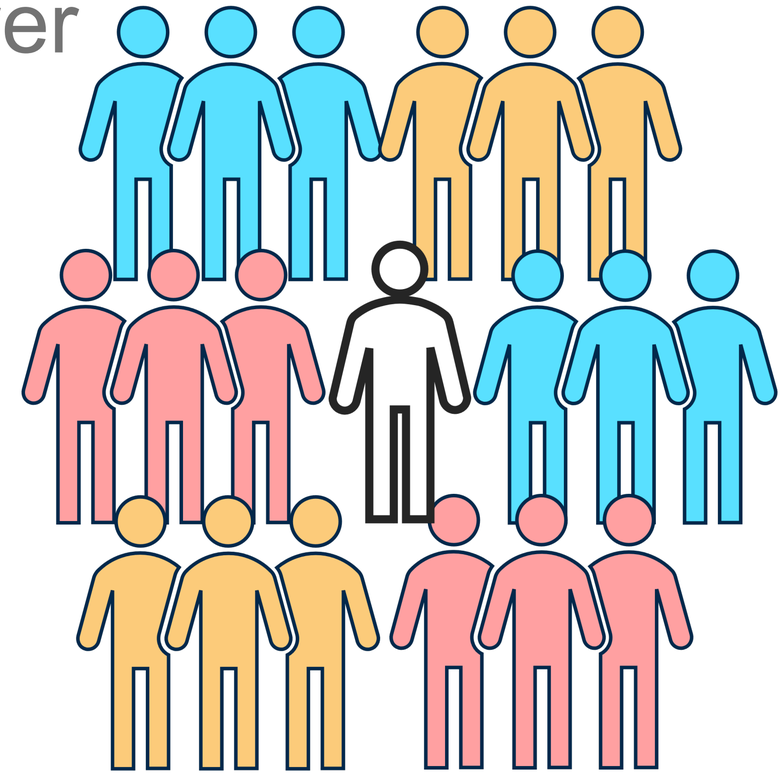
- Wheeler, 3 years old, Juvenile Batten Disease
- Will rob him of eyesight, speech, mobility, life
- Currently no treatment or cure
- miglustat (used for Gaucher Disease) may slow progression, ***but:***
  - Drug costs $24,000 / month
  - Health insurances won't cover unapproved use
  - Pediatric clinical trial is one way to get the drug, but not scheduled until 2024
  - Wheeler's parents are appealing coverage, but time is running out



https://www.statnews.com/2023/02/28/fda-needs-to-build-in-more-flexibility-for-rare-disease-clinical-trials/

What is an orphan disease?

- Definition: single disease impacting fewer than 200,000 patients in US
- 7,000+ known rare diseases (11,000?)
- Altogether affect 1 in 10 Americans
- Often severe in nature
- Typically affect very young, vulnerable populations



Agenda

Time (PM EST)	Topic	Speaker	Background
1:45-1:50	Overview	Meg Richards, PhD, MPH	Exec Director, Solutions, Panalgo Boston, MA
1:50-2:05	Current landscape of health plan coverage for rare diseases in the US	James D. Chambers, PhD, MPharm, MSc	Associate Professor Tufts Medical Center Boston, MA
2:05-2:20	Established framework(s) for ‘fair’ access	Sarah K. Emond, MPP	EVP & COO ICER Boston, MA
2:20-2:35	Challenges to access from the patient perspective	Sneha Dave, BS	Founder & Executive Director Generation Patient Indianapolis, IN
2:35-2:45	Q&A	Meg Richards, PhD, MPH	Exec Director, Solutions, Panalgo Boston, MA

TuftsMedicine

Orphan Drug Fair Access

James Chambers PhD

The orphan drug challenge

- Orphan drugs cost 5x more than non-orphan drugs
- >10% of prescription drug spending (4% in 1997)
- From 2016 through 2022, 50.5% (159 of 315) of novel FDA drug approvals were orphan drugs*

* U.S. Food and Drug Administration. New Drugs at FDA: CDER's New Molecular Entities and New Therapeutic Biological Products. 01/10/2023

SPEC DATABASE

Specialty Drug Evidence & Coverage

- ✓ 18 large US commercial health plans
- ✓ 190+ million covered lives
- ✓ 388 specialty drugs
- ✓ 150+ diseases
- ✓ 100+ biopharma companies
- ✓ 75,000+ coverage decisions
- ✓ 60,000+ citations

United Healthcare

Blue Cross Blue Shield Blue Care Network of Michigan

Humana

Kaiser Permanente

Independence

Highmark Health

aetna

Centene Corporation

Anthem Blue Cross

HCSC Health Care Service Corporation

BlueCross BlueShield of North Carolina

EmblemHealth

Horizon Blue Cross Blue Shield of New Jersey

Massachusetts

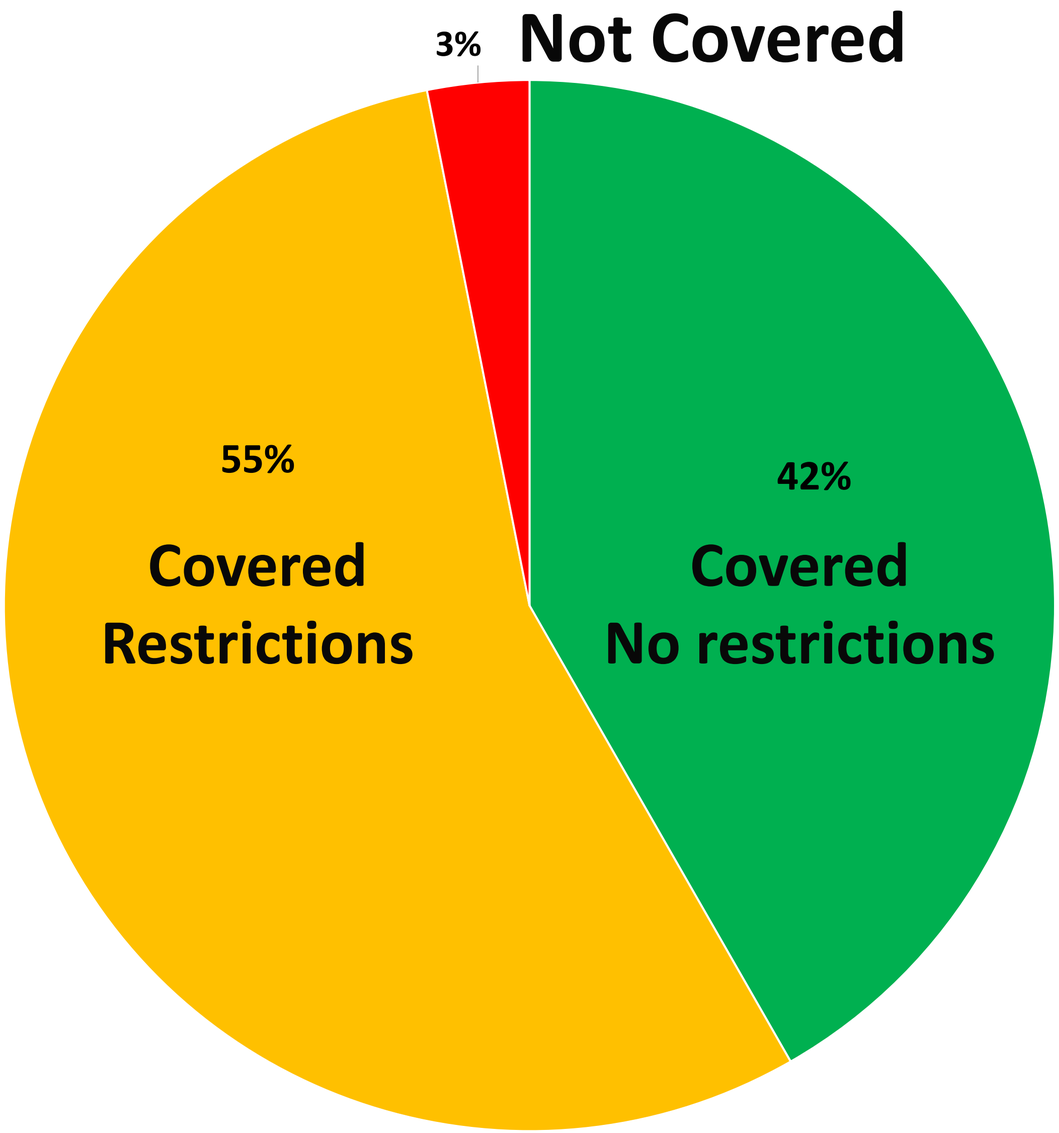
Cigna

Florida Blue

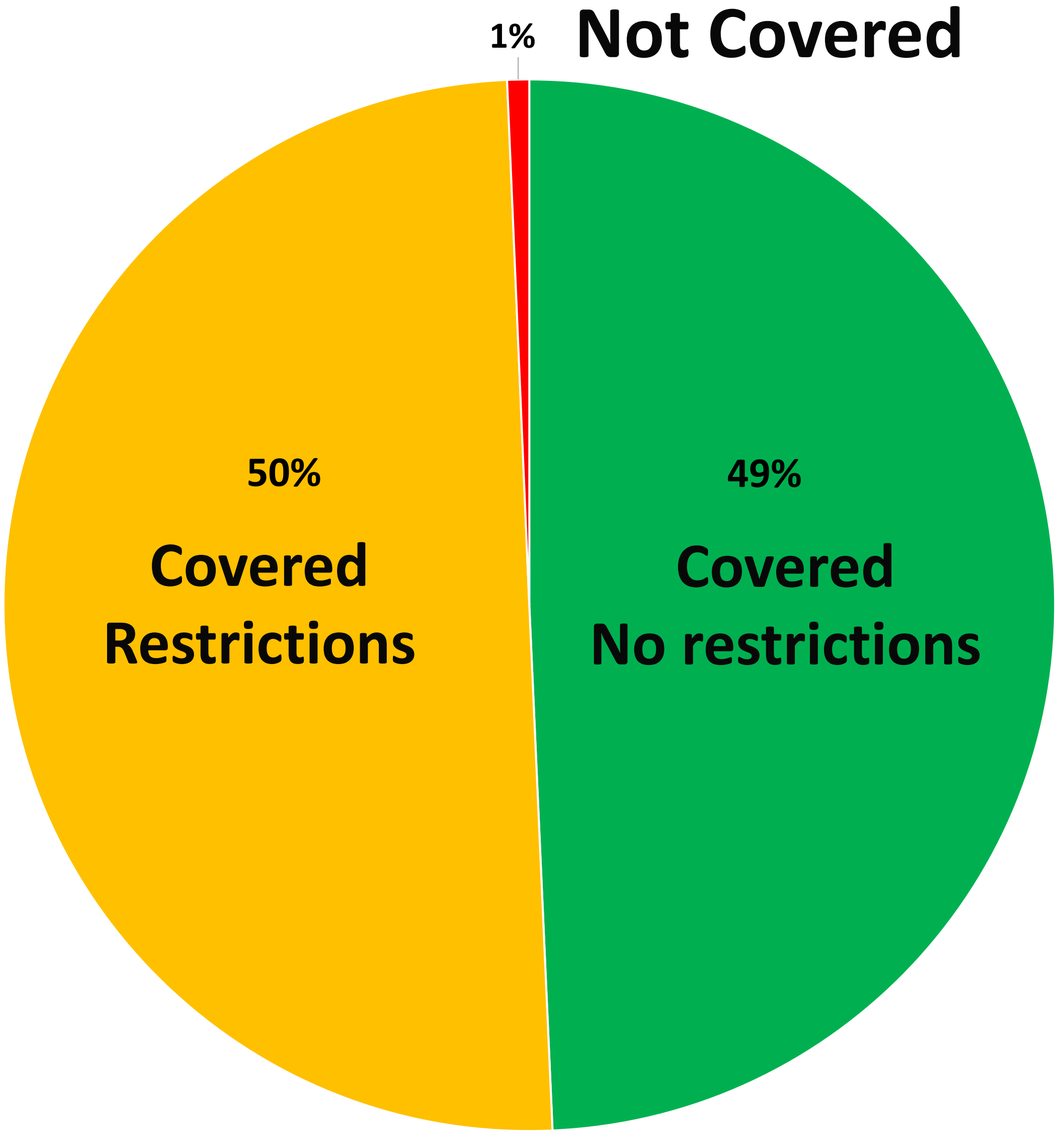
CareFirst BlueCross BlueShield

of Tennessee

Non-orphan drugs (n=6,234)

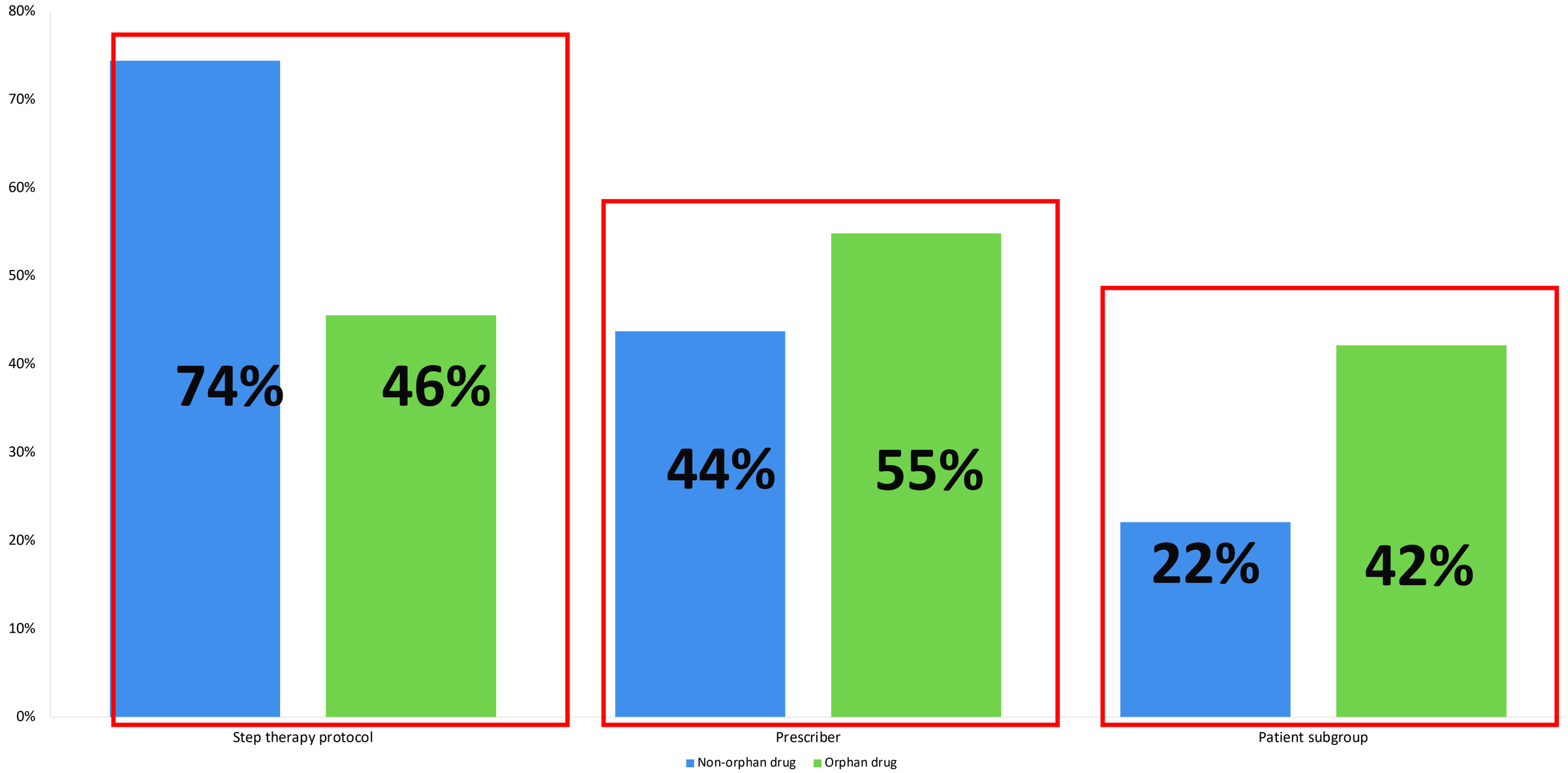


Orphan drugs (n=5,045)



* Source. Tufts Medical Center. Specialty Drug Evidence and Coverage (SPEC) Database. Accessed April 17th 2023. Available: https://cevr.tuftsmedicalcenter.org/databases/spec-database

Coverage restriction types: Non-orphan vs. orphan



* Source. Tufts Medical Center. Specialty Drug Evidence and Coverage (SPEC) Database. Accessed April 17th 2023. Available: https://cevr.tuftsmedicalcenter.org/databases/spec-database

Commercial health plans use of patient subgroup restrictions: An analysis of orphan and FDA-expedited programs

Nola B Jenkins, BA; Julia A Rucker, MSW/MPH; Alexa C Klimchak, MA; Lauren E Sedita, BS; James D Chambers, PhD

Plain language summary

Health plans may manage patients' eligibility for a drug by requiring that the patients meet certain clinical criteria (eg, symptoms that indicate disease severity or being within a specified age range). These are known as patient subgroup restrictions. Plans imposed patient subgroup restrictions in roughly one-fifth of orphan drug coverage policies and in roughly one-fifth of US Food and Drug Administration–expedited drug policies. Patient subgroup restrictions were typically consistent with the data the US Food and Drug Administration reviewed to grant approval.

Implications for managed care pharmacy

US commercial health plans impose patient subgroup restrictions in their drug coverage policies with different frequencies, indicating that a patient's plan can greatly influence their access to care. Patient subgroup restrictions often used the same clinical measures as included in drugs' pivotal clinical trials. However, patient subgroup restrictions were inconsistent with the pivotal trial's eligibility criteria roughly one-fifth of the time, raising questions about how health plans developed those criteria.

Author affiliations

Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center, Boston, MA (Jenkins, Rucker, Chambers); Sarepta Therapeutics, Inc., Cambridge, MA (Klimchak and Sedita).

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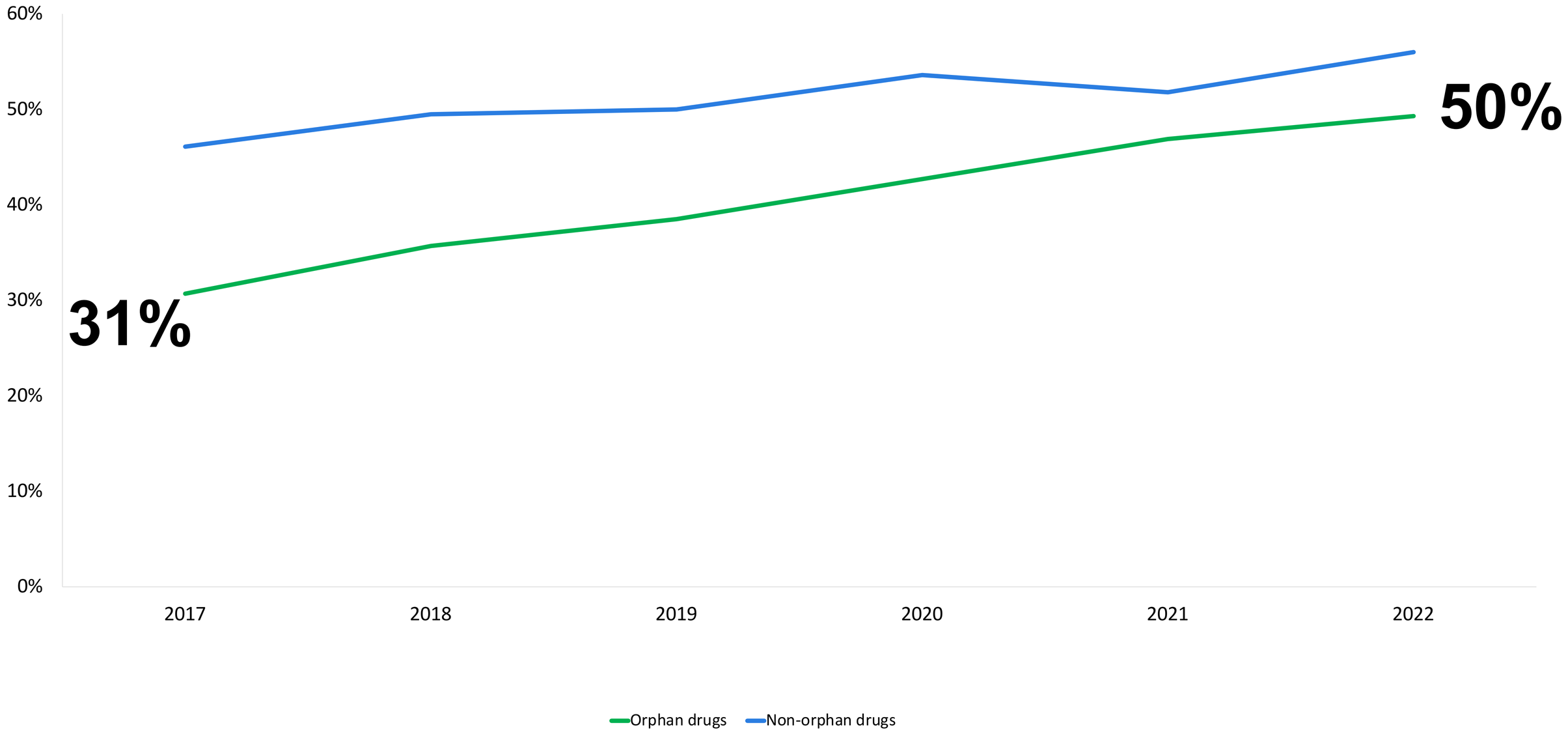
J Manag Care Spec Pharm.
[Published online March 8, 2023]

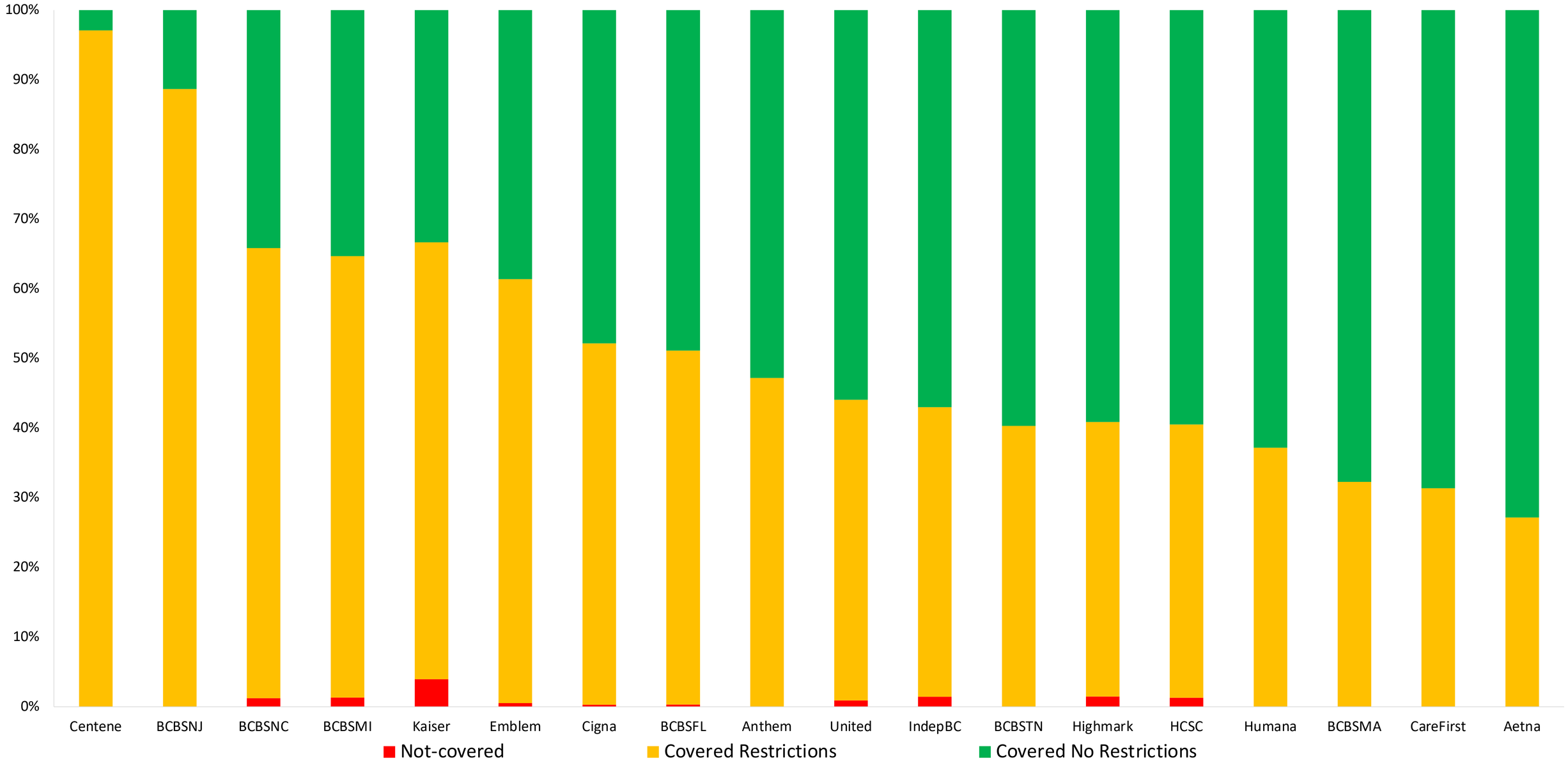
Orphan drug coverage restrictiveness over time



* Source. Tufts Medical Center. Specialty Drug Evidence and Coverage (SPEC) Database. Accessed April 17th 2023. Available: https://cevr.tuftsmedicalcenter.org/databases/spec-database

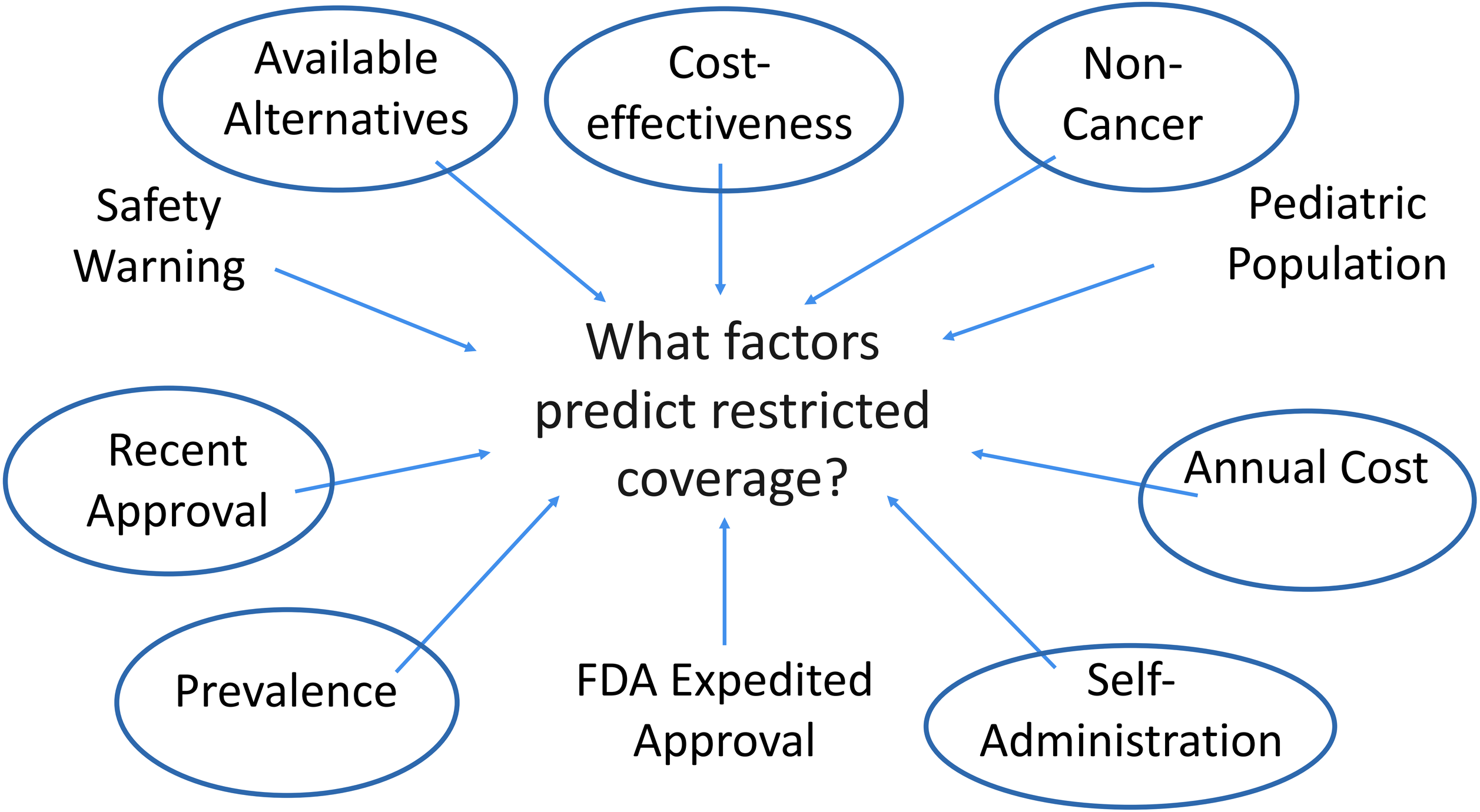
Do all health plans cover orphan drugs the same way?

Variation in orphan drug coverage



* Source. Tufts Medical Center. Specialty Drug Evidence and Coverage (SPEC) Database. Accessed April 17th 2023. Available: https://cevr.tuftsmedicalcenter.org/databases/spec-database

Under what circumstances do payers restrict orphan drug coverage?


Available Alternatives
Cost-effectiveness
Non-Cancer
Safety Warning
Pediatric Population
What factors predict restricted coverage?
Recent Approval
Annual Cost
Prevalence
FDA Expedited Approval
Self-Administration

Plans are more likely to apply restrictions when…

1. Non-cancer
2. Available alternative(s)
3. Recent approval
4. Higher annual cost
5. Larger prevalence
6. Less favorable cost-effectiveness

PharmacoEconomics (2022) 40:225–232
https://doi.org/10.1007/s40273-021-01096-5

ORIGINAL RESEARCH ARTICLE

Is an Orphan Drug's Cost-Effectiveness Associated with US Health Plan Coverage Restrictiveness?

James D. Chambers[1] · Nikoletta M. Margaretos[1] · Daniel E. Enright[1] · Rosa Wang[2] · Xin Ye[2]

Accepted: 19 September 2021 / Published online: 26 October 2021

Abstract

Background and Objectives Orphan drugs' high prices raise questions about whether their costs are worth their benefits. We examined the association between an orphan drug's cost-effectiveness and health plan coverage restrictiveness.

Methods We analyzed a dataset of US commercial health plan coverage decisions (information current as of 2019) for orphan drugs ($n = 3298$). We used multi-level random-effect logistic regression to examine the association between orphan drug cost-effectiveness and coverage restrictiveness. We identified cost-effectiveness estimates from the Tufts Medical Center Cost-Effectiveness Analysis Registry, and from the Institute for Clinical and Economic Review's value assessments. We included only cost-effectiveness studies not funded by product manufacturers. We included the following independent variables: cancer indication, availability of alternatives, pediatric population, number of years since US Food and Drug Administration (FDA) approval, disease prevalence, annual cost, additional non-orphan indication, safety, and inclusion in a FDA expedited review program.

Results Plans restricted drug coverage in 29.7% ($n = 981$) of decisions. Plans were more likely to restrict drugs with incremental cost-effectiveness ratios of \$50,000–\$175,000 per quality-adjusted life-year [QALY] (odds ratio = 1.855, $p < 0.05$), \$175,000–\$500,000 per QALY (odds ratio = 1.859, $p < 0.05$), and >\$500,000 per QALY/dominated (odds ratio = 2.032, $p < 0.01$), compared to drugs with incremental cost-effectiveness ratios <\$50,000 per QALY. Plans more often restricted drugs with non-cancer indications, having available alternatives, with more recent approval, in an FDA expedited review program, and for which the FDA additionally issued approval for a non-orphan disease. Plans more often restricted drugs with higher annual costs, and drugs indicated for higher prevalence diseases. All findings $p < 0.05$.

Conclusions Among other factors, an orphan drug's cost-effectiveness was associated with health plan drug coverage restrictiveness.

Examining US commercial health plans' use of The Institute for Clinical and Economic Review's reports in specialty drug coverage decisions

James D Chambers, PhD; Daniel E Enright, BA; Ari D Panzer, BS; Josh T Cohen, PhD; Daniel A Ollendorf, PhD; Peter J Neumann, ScD

Plain language summary

The Institute for Clinical and Economic Review (ICER) has a prominent role in US health care. We found an association between ICER's value assessments and how US commercial health plans cover specialty drugs. We also found that although health plans rarely cite ICER's assessments, they are doing so more frequently.

Implications for managed care pharmacy

We found that, when controlling for other decision-making factors (eg, orphan drug status, availability of alternatives), US commercial health plans' specialty drug coverage decisions were associated with ICER's value assessments. In other words, plans tended to impose fewer coverage restrictions on drugs that ICER estimated to have more favorable cost-effectiveness ratios. Future research should examine plans' responsiveness to ICER's value assessments and how plans use this information in drug coverage policy formulation.

Author affiliation

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2023;29(3):257-64

Key Findings

- One third of orphan drug coverage decisions restricted
- Orphan drug coverage becoming more restrictive
- Variation in health plan behavior
- Cost-effectiveness associated with orphan drug coverage

Thank you!

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What Is ‘Fair’ Access for Orphan Drugs in the US?

Presentation at ISPOR 2023

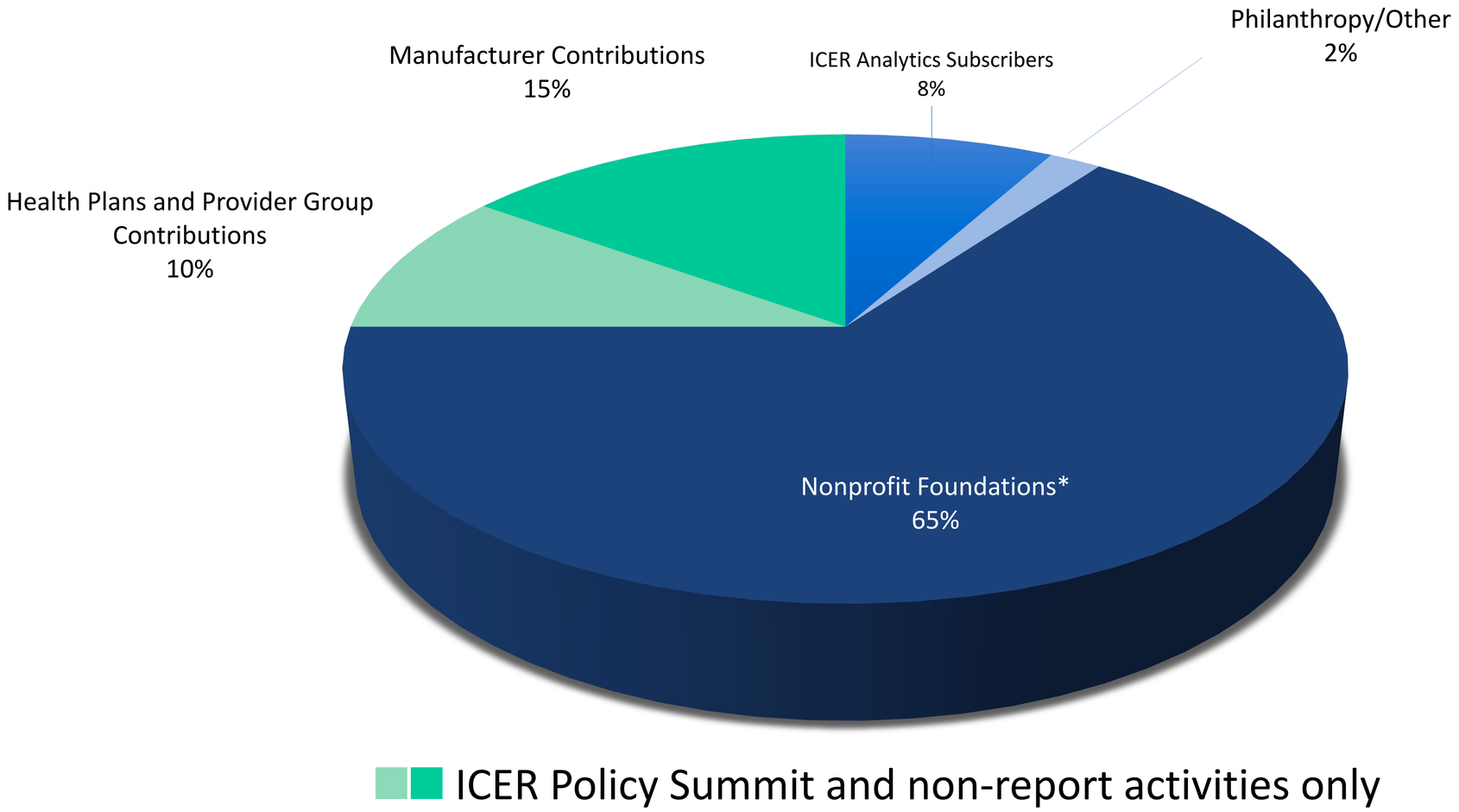
Sarah K. Emond, MPP

ICER
INSTITUTE FOR CLINICAL
AND ECONOMIC REVIEW

Institute for Clinical and Economic Review (ICER)

- **Independent, non-partisan** health technology assessment group whose reviews are funded by non-profit foundations
- Develop **publicly-available value assessment reports** on medical tests, treatments, and delivery system innovations for nearly 15 years
- Convene regional independent **appraisal committees** for public hearings on each report
- For some analyses, use cost-effectiveness analysis to determine **health benefit price benchmarks**
- Produce annual list of Unsupported Price Increases using **comparative clinical effectiveness** expertise
- Annual **“Fair Access”** report examining whether insurers are providing fair access to drugs

Funding 2023



https://icer.org/who-we-are/independentfunding/sources-of-funding/

*ICER received significant funding from Arnold Ventures, The California Health Care Foundation, and The Commonwealth Fund

Fair Pricing.

Fair Access.

Future Innovation.

CORNERSTONES OF "FAIR" DRUG COVERAGE: APPROPRIATE COST-SHARING AND UTILIZATION MANAGEMENT POLICIES FOR PHARMACEUTICALS

September 28, 2020

Cornerstones of Fair Drug Coverage: White Paper

- White paper recommends appropriate policies that determine patient access to prescription drugs
- Informed by expert input from patient groups, clinician specialty societies, payers, and life science companies
- Categories include: cost-sharing, clinical eligibility criteria, step therapy, prescriber restrictions

Criteria for Fair Drug Coverage

- Clinical eligibility/Narrowing the FDA label:
  - Clinical eligibility criteria that complement the FDA label language may be used to:
    - Set standards for diagnosis;
    - Define indeterminate clinical terms in the FDA label (e.g., “moderate-to-severe”)
  - For drugs not fairly-priced: Clinical eligibility criteria may narrow coverage by applying specific eligibility criteria from the pivotal trials used to generate evidence for FDA approval
- Cost-sharing:
  - At least one drug in every class should be covered at the lowest relevant cost-sharing level unless all drugs are priced higher than an established fair value threshold.

Criteria for Fair Drug Coverage

- Provider Restrictions:
  - Restrictions of coverage to specialty prescribers are reasonable when:
    - Accurate diagnosis and prescription require specialist training, with the risk that non-specialist clinicians would prescribe the medication for patients who may suffer harm or be unlikely to benefit.
    - Determination of the risks and benefits of treatment for individual patients requires specialist training due to potential for serious side effects of therapy.
    - Dosing, monitoring for side effects, and overall care coordination require specialist training to ensure safe and effective use of the medication.

Barriers to Fair Access: Annual Report

- Multi-stakeholder working group guides assessment of major payer policies:
  - January 2023: second annual report on how policies align w/ fair access criteria
- Most available payer policies were structured appropriately to support many key elements of fair access
- Five payers improved tiering or coverage of 11 policies after ICER shared draft results
- Report calls for greater transparency regarding how insurers communicate cost-sharing and clinical criteria to prospective members

Orphan Drug Fair Access

- 13 drugs across two years were orphan drugs; 6 were fairly-priced
- High concordance rates with fair access criteria:
  - Cost-sharing: 56-100%
  - Clinical Eligibility: 70-100%
  - Step Therapy: 82-100%
  - Provider Restrictions: 100%
- Not correlated with being fairly-priced; not significantly different than non-orphan drugs
- Limitations on the types of criteria we could measure, so generalizations are not possible

Examples of Policies Not Meeting Fair Access Criteria

- Requiring patients to have a history of bleeding or joint damage before getting access to Hemlibra® (emicizumab for hemophilia A)
  - Inconsistent with FDA label or clinical guidelines (*one payer updated policy after reviewing our draft results*)
- Requiring a certain functional status to be eligible for CAR-T therapies (leukemia and lymphoma)
  - Stricter than the FDA label
- Not having Lynparza® (olaparib for ovarian cancer), Haegarda® (C1 esterase inhibitor for hereditary angioedema), Hemlibra on the lowest relevant tier
  - Fairly-priced drug or alternative must be on lowest tier (*two payers updated cost-sharing after reviewing our draft results*)

What Is ‘Fair’ Access for Orphan Drugs in the US?

Presentation at ISPOR 2023

Sarah K. Emond, MPP | semond@icer.org

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Patient Perspectives

2023 ISPOR Orphan Drug Fair Access

GenerAtion patient

Generation patient

Events

HAS
HEALTH ADVOCACY SUMMIT

The Health Advocacy Summit is the prior name of the overall organization but now serves as the name for our virtual and in-person events. Prior to the pandemic, we facilitated seven in-person Summits in four states and during the pandemic, we have facilitated three international virtual Summits.

Programs

CCYAN
Crohn's and Colitis Young Adults Network

The Crohn's and Colitis Young Adults Network (CCYAN) facilitates a fellowship program, community space, and more for young adults with Inflammatory Bowel Diseases. CCYAN is the only disease-specific programming of Generation Patient. Visit www.ccyanetwork.org.

(HEALTH) polic la y b

The mission of the (Health) Policy Lab is to provide health policy education and advocacy opportunities to young adults with chronic and rare conditions in an effort to increase meaningful access to prescription medications. Visit www.hplab.org.

& More!

- 6 Virtual Meetings per month
- Roundtables to bring together stakeholders on a variety of topics
- Advocacy to increase access to higher education
- Critical resources for our community, including civic engagement and advance care planning.

Disclosures

Our support comes from foundations such as the Helmsley Charitable Trust, Arnold Ventures, the Commonwealth Fund, the Disability Inclusion Fund, Third Wave Fund, and the Lumina Foundation.

We are independent of all private healthcare industry funding.

Patient Perspectives

To me fair access to orphan drugs for rare diseases means being able to have confidence that the drug that I rely on for survival will always be available to me. Availability in terms of supply, but also available in terms of affordability. I am currently dealing with the effects of a national shortage for Solu-Cortef (powder vials of hydrocortisone for my cortisol pump). Not knowing how I will fill this life saving and life sustaining medication is beyond frightening. In addition, ensuring that drug prices remain affordable and stable will contribute to that fair access.

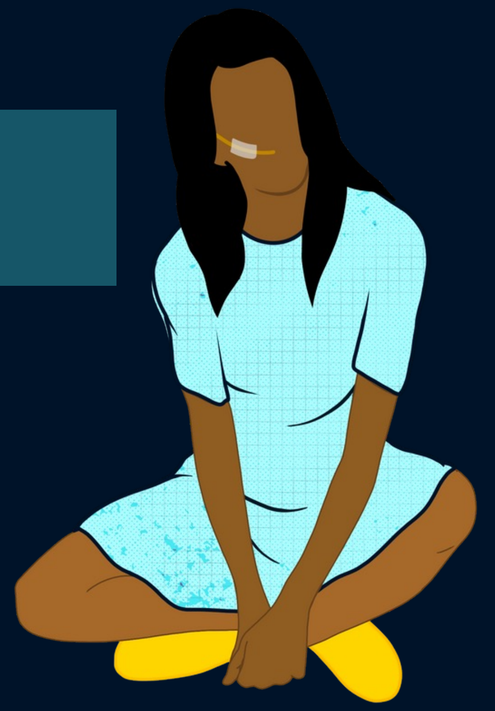
I think it's only fair that pharmaceutical manufacturers are held accountable for fair access to their products. It must always be remembered that what is "just another drug" to them is my lifeline. What is just another source of income to them is the only thing keeping me alive. There should be awareness that they hold my life in their hands and treat that with the appropriate care.

Mara Shapiro, rare disease patient, age 23 with her dog Morty.

A reality of our healthcare system

Patients do not have time or energy to spend navigating macro-level challenges that occur between regulatory bodies, payers, and pharmaceutical companies.

The reality: Our society is not set up to pay for ultra high-cost drugs.



Contextual Considerations

What is fair?

- International pricing
- Number of patients
- Life-threatening or not
- Out-of-pocket cost
- Research and development costs
- Pediatric versus adult patients
- Cure versus treatment of symptoms

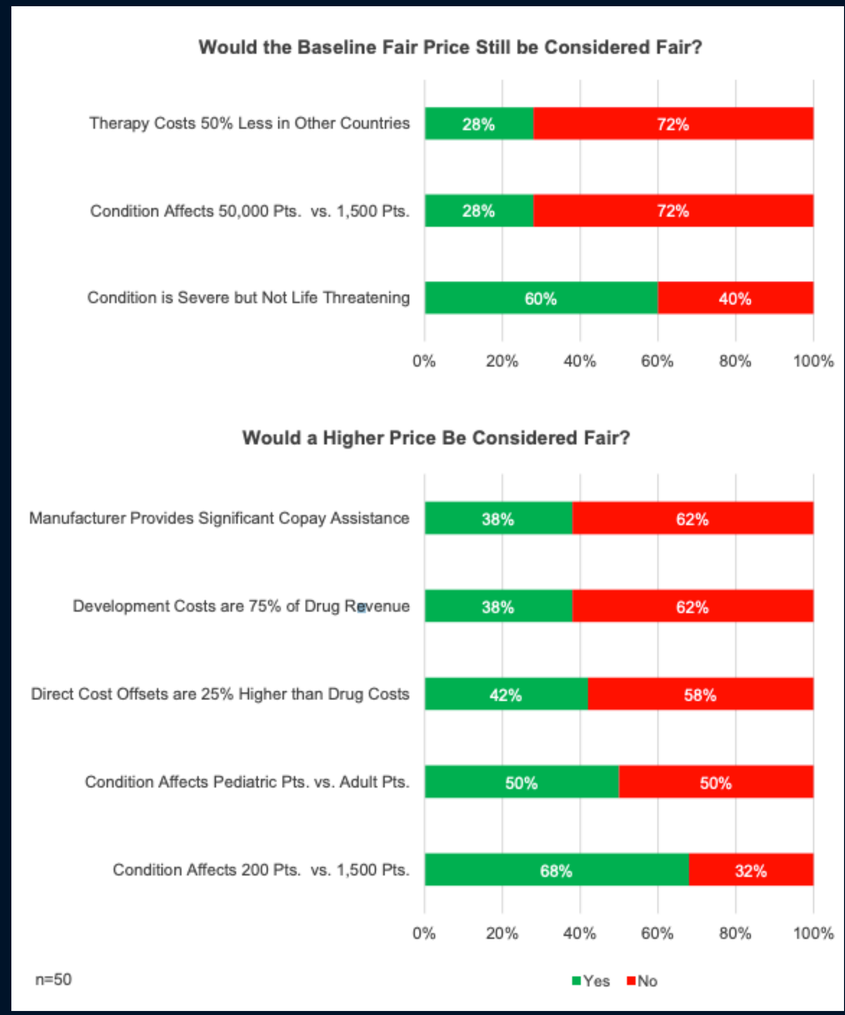
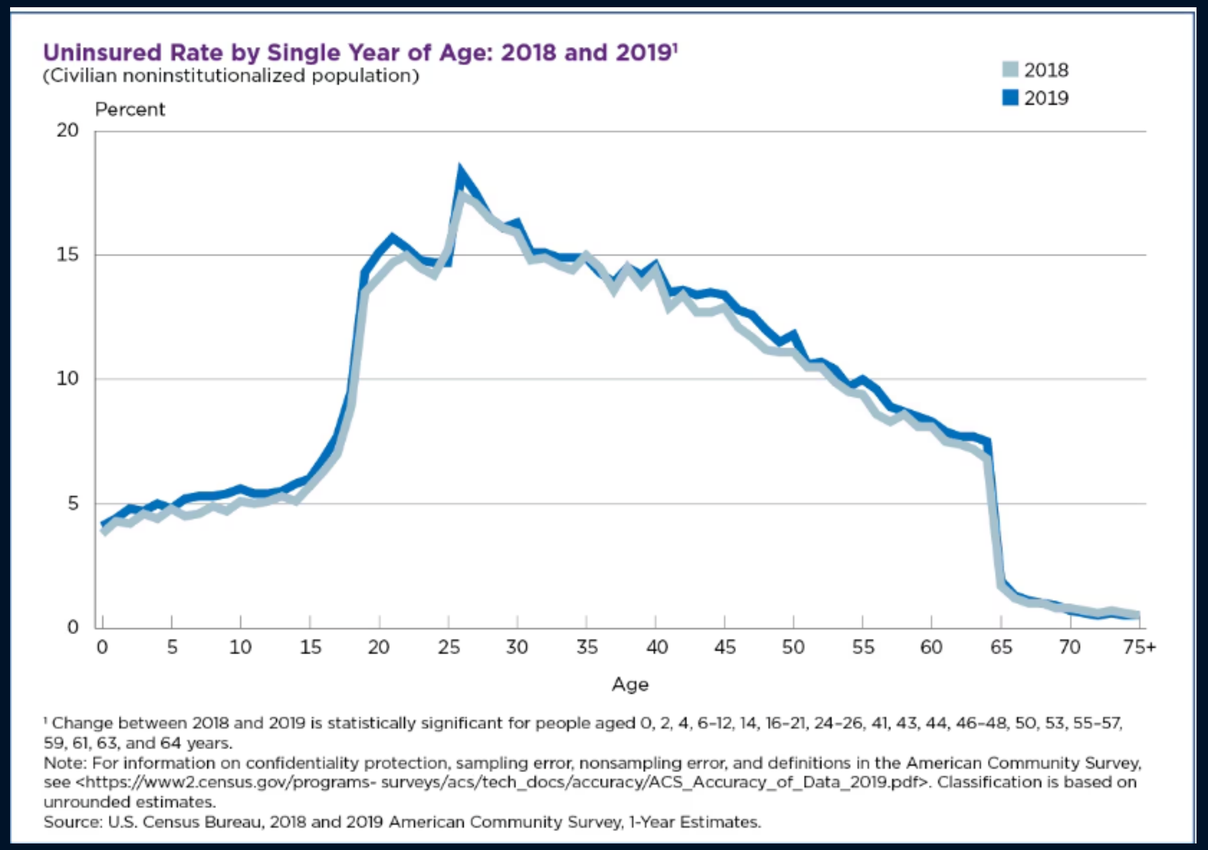


Image from Charles River Associates "Pricing for rare disease and curative therapies: What's fair?"

Charles River Associates

Adolescents and Young Adults

- The transition of insurance burden and loss of continuity of care.
- Access to orphan drugs might depend on health plans which might restrict location mobility.



Adults ages 19 to 34 had the highest uninsured rates of any age group in the United States, according to the 2019 American Community Survey (ACS).

Census.gov

Off-label Usage & Cost

Cost-sharing for off-label use:

- Time between approval for off-label use and need for prescription drug
- Urgency of off-label usage of prescription medications may result in greater direct patient cost

Abbey's story

"I was prescribed an off-label medication in my home state and it was covered by my insurer with a smaller co-pay. But as I moved to another state for graduate school, I had to pay out of pocket for the medication for a few months."

Abbey Hauser, young adult rare disease patient

Patient Cost

Cost-sharing

- Out of pocket maximums
- Copayments
- Coinsurance

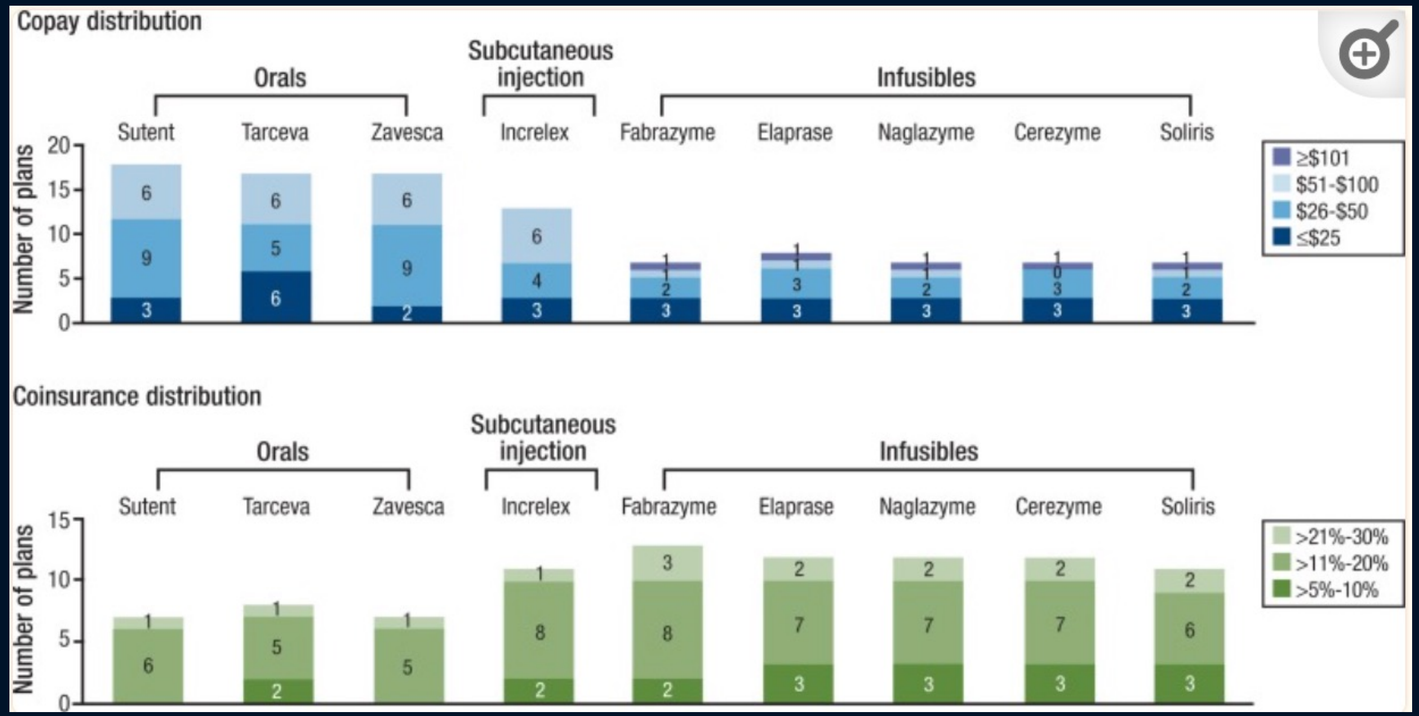
Affordability challenges

- Rising premiums
- Deductibles
- Coverage based on plan type
- Limits on benefits

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4106547/

Patient Assistance

- Patient assistance programs are not available for everyone.
  - Often will be for patients unable to afford insurance or cost-sharing.



Source: InVentiv Advance Insights, Somerset, NJ.

Young adult patient perspective

For more than ten years, my quality of life has depended on monthly infusions which, without insurance, would cost me over $10,000 a month to receive. To receive these infusions and also access the healthcare team that has been managing my care for over ten years, I have to pay almost $800 a month for private insurance. I'm grateful that I'm able to do so, but it's a huge financial burden for someone who has only been gainfully employed for two years. Before that, I had to rely on my parents for help either by paying for my insurance costs or by having me on the insurance provided by their employer. This put a lot of pressure on my family. For a long time, my mom was afraid to leave a company that did not treat her well at all because she was afraid of losing her benefits and the impact that would have on me.

Anonymous, young adult patient

Considerations

- Ancillary, administrative, and time costs
- Comorbidities
- Multiple diagnoses just for approval of the therapeutic instead of one rare, primary diagnosis to get approval for orphan drugs

What is important to patients

- What is considered novel?
  - Deepening vs. divergent innovation
- What do patients want?
  - Depends, but how can deepening innovation lead to drug repurposing and fewer "me-too" drugs?

Patents
"Not new enough"

VS.

Payers and Regulatory agencies
"Too New"

"The Cost of Novelty" W. Nicholson Price II

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Thank You!

Let’s Discuss!

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