# Patient Access to Hemophilia A Treatment Varies Across US Commercial Health Plans

PRO53

**Margaretos N.M**[1], Patel A.M[2], Panzer A.D[1], Khairnar R[2], Chambers J.D[1]

[1] Center for the Evaluation of Value and Risk in Health, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center, Boston, MA, USA

[2]Genentech, Inc., San Francisco, CA, USA

Tufts Medical Center

Proudly wellforce

**PUSHED FOR TIME?**

In order to use the QR code, please use or download an app called 'QR code reader' from the Apple Appstore or the Android Playstore.

Receive an instant copy of this poster: https://bit.ly/3f5AFjN

CEVR Center for the Evaluation of Value and Risk in Health

## BACKGROUND AND OBJECTIVE

- Health plans formulate their own coverage decisions, hence how they cover specialty products can vary.[1] This variation can have important implications for patients' access to care.
- Hemophilia A is a genetic disorder caused by missing or defective factor VIII (FVIII), which is an essential blood-clotting protein.[2]
  - Hemophilia A is a rare disease that is associated with notably high treatment costs.
- In this study, we examine how large US commercial health plans cover hemophilia A treatments compared to FDA labeled indications.

## METHODS

### Data Source

- The starting point for this research was the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) Database, which contains publicly available specialty product coverage policies issued by 17 of the largest US commercial health plans.
- We identified coverage decisions for hemophilia A treatments in the SPEC Database. When a hemophilia A treatment was not included in SPEC, we searched for and obtained the coverage policies from the health plans' websites.
- Our sample included 26 hemophilia A treatments: one non-factor antibody, three bypassing agents, two desmopressin products, five plasma-derived FVIII products, and fifteen recombinant FVIII products.
- Coverage decisions were current as of August 2019.

### Coverage Restrictiveness

- We compared each coverage decision with the treatment's FDA labeled indication. We categorized decisions as:
  - Less restrictive than the FDA labeled indication: the plan covers a broader population than the FDA labeled indication
  - Equivalent to the FDA labeled indication
  - More restrictive than the FDA labeled indication: the plan placed conditions on coverage beyond those in the FDA indication
  - Mixed coverage restrictiveness: the plan covers more restrictively than the FDA labeled indication in one way, but less restrictively in another
  - Not covered: the plan does not cover the treatment.

### Restriction Types

- We considered a coverage decision to be more restrictive than the drug's FDA label if a health plan applied one or more of the following restriction types:
  - Step therapy protocol: the plan requires the patient to first fail an alternative treatment before gaining access to the drug
  - Patient subgroup restriction: the plan requires patients to meet particular clinical criteria (e.g., documentation of history of ≥2 spontaneous bleeds into joints)
  - Other restrictions: the plan applies any other types of coverage restrictions.

## RESULTS

- Sixteen of the 17 health plans issued publicly available coverage policies for hemophilia A treatments (n=297 coverage decisions).
- We classified 48% of coverage decisions as 'more restrictive', 7% as 'less restrictive', 37% as 'equivalent', 7% as 'mixed', and <1% as 'not covered'.
- In restricted decisions, plans most frequently applied patient subgroup restrictions (82% of decisions). The majority of patient subgroup restrictions were bleeding related, e.g., a requirement that patients have at ≥2 documented episodes of spontaneous bleeding into joints.

## RESULTS CONTINUED

- Health plans applied step therapy protocols in 29% of restricted decisions. For example, one plan required that patients with mild hemophilia (i.e., factor VIII activity level between 5–40%) try and fail desmopressin acetate before being able to access emicizumab.

### Variation Across Health Plans

- Plans varied in the frequency that they applied coverage restrictions, ranging from 4 to 100% of their decisions (**Figure 1**).
- Two plans applied restrictions in 100% of their coverage decisions; ten plans applied restrictions in at least half of their decisions.

### Variation Across Hemophilia A Treatments

- Health plans covered some hemophilia A treatment classes more restrictively than other classes (**Figure 2**).
- No treatment was covered consistently across all 16 plans, i.e., some plans applied coverage restrictions, while other plans did not.
- For 19 of the 26 included treatments, at least half of health plans applied restrictions in their coverage decisions.
- Plans most often applied restrictions in their coverage decisions for the single non-factor antibody, emicizumab, in our sample (10/15 decisions). All ten plans applied patient subgroup restrictions (which 60% of the time were bleeding related) and four plans additionally applied step therapy protocols. Plans tended to cover emicizumab more restrictively for patients without FVIII inhibitors than for patients with FVIII inhibitors.
  - Three plans did not cover emicizumab for patients without FVIII inhibitors.
  - Three plans covered emicizumab more restrictively for patients without FVIII inhibitors than for patients with FVIII inhibitors, e.g., one plan required documentation of clinically evident bleeding after a 6 month trial of ≥3 factor VIII products for patients without FVIII inhibitors, but did not impose the same requirement on patients with FVIII inhibitors.

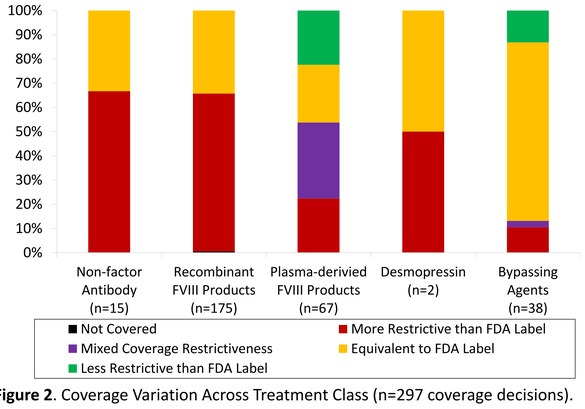


**Figure 2**. Coverage Variation Across Treatment Class (n=297 coverage decisions).
Note: (n=) refers to the number of decisions issued for all treatments in the class.

## CONCLUSIONS

- Overall, US commercial health plans applied restrictions in more than half of their coverage decisions for hemophilia A treatments.
- Some health plans covered particular treatments more restrictively for patients without FVIII inhibitors than for patients with FVIII inhibitors.
- Variation across health plan coverage decisions suggest that a patient's plan may influence their access to hemophilia A treatments.

## REFERENCES

1. Chambers JD, *et al. Health Aff (Millwood)* 37(7):1041–7.
2. Mannucci PM, Tuddenham EG. *New Engl J Med* 2001;344:1773–9.

## FUNDING

Genentech, Inc. funded this research.

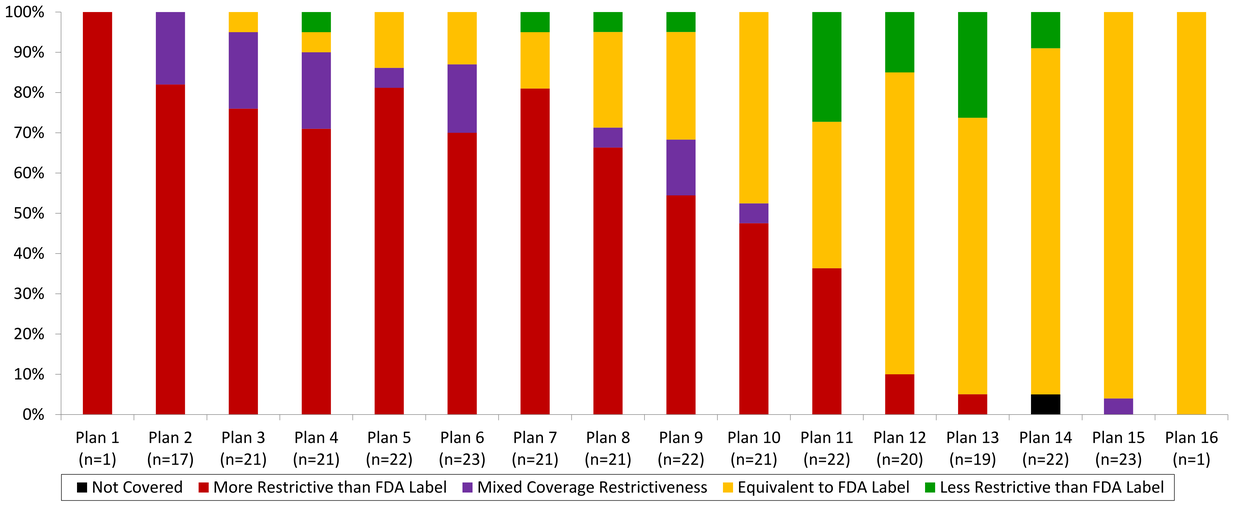


**Figure 1**. Coverage Variation Across Plans (n=297 coverage decisions). Note: (n=) refers to the number of decisions issued by each health plan.