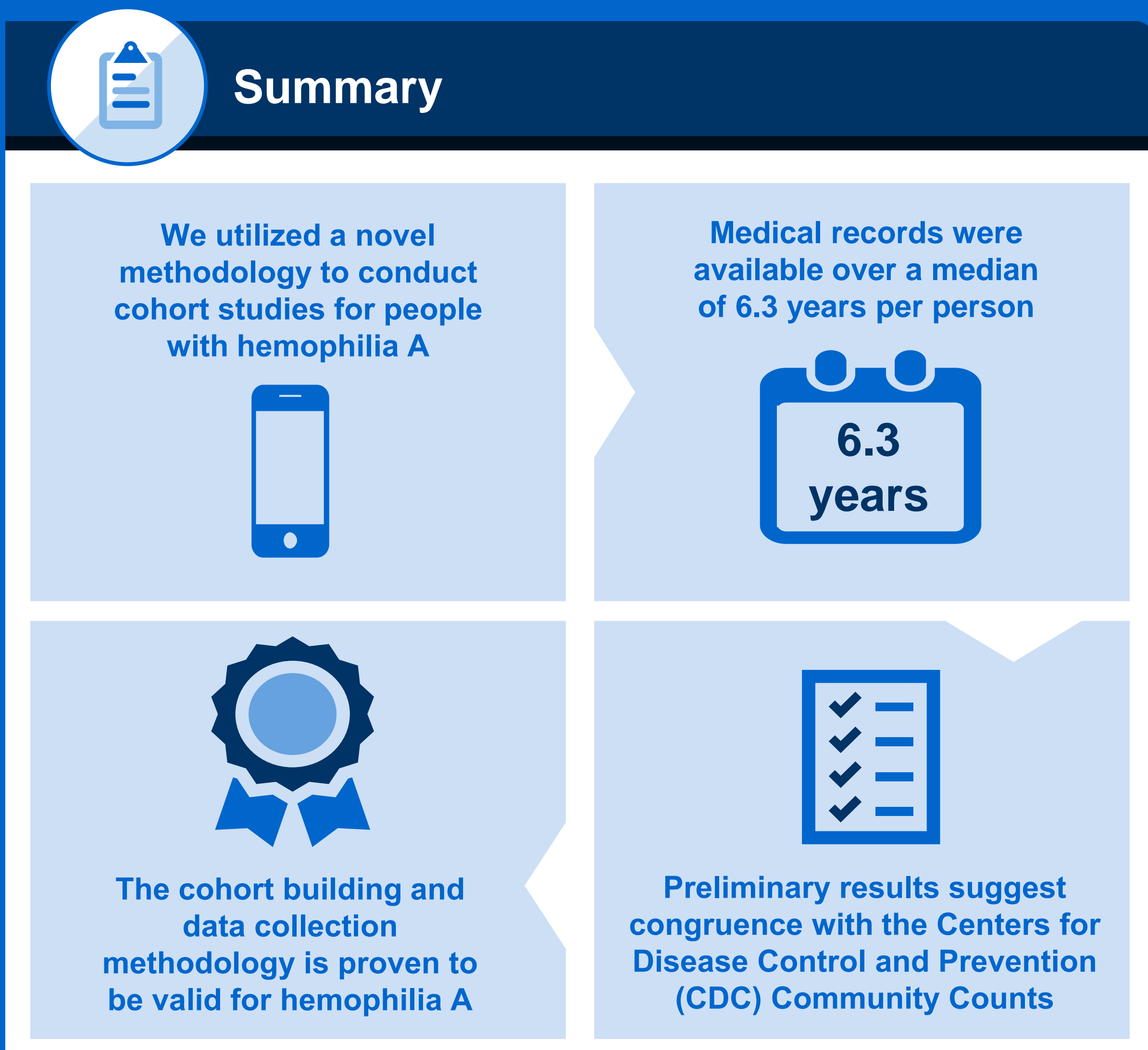


# Characterizing Mild and Moderate Hemophilia A Patients in the Real World: A Patient-Centric Approach

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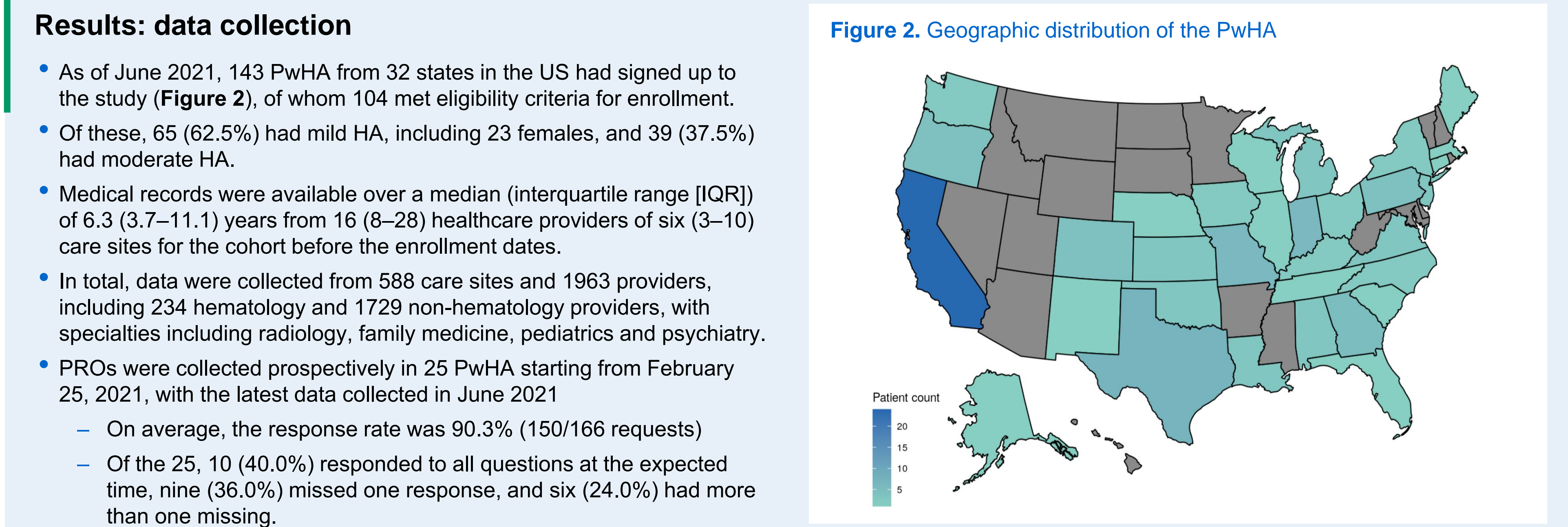
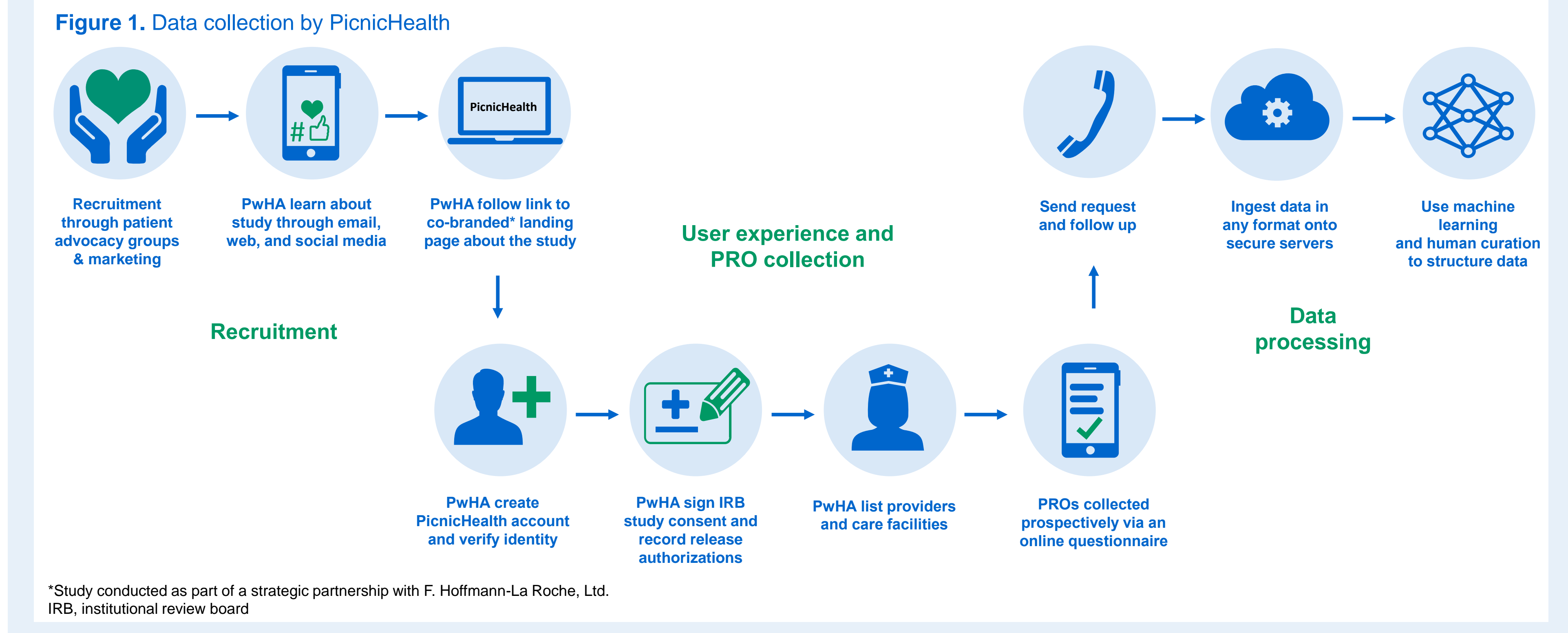
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## Background

- People with mild or moderate hemophilia A (HA) account for 40–52% of all people with HA (PwHA) but are under-represented in scientific research.<sup>1,2</sup>
- To address this gap, we utilized a novel, patient-centric approach developed by PicnicHealth to create a longitudinal healthcare database from people with mild or moderate HA in the United States (US).
- This study integrates data collected during routine clinical care from various providers and sites along with patient-reported outcomes (PROs) on an online record management platform to provide an in-depth characterization of patients' disease journey.

- ### Methods
- PicnicHealth HA study recruitment commenced in June 2020 following ethics approval. Consented eligible PwHA had a documented HA diagnosis.
  - Severity status was confirmed based on physician-reported severity in provider notes or reported baseline factor (F)VIII level (>5–40% mild, 1–5% moderate); the lowest FVIII value was used if notes and baseline levels were inconclusive.
  - Data elements from structured text (e.g. medication lists), as well as disease-specific elements from narrative text, were captured from patients' electronic health records and linked to self-reported data (**Figure 1**). PROs, including bleeds, treatment, physical and social activity, and pain, were collected biweekly via online questionnaire for a subset of PwHA.
  - Quality control was assessed via inter-abstractor agreement on outputs with physician review. Descriptive analyses were performed to summarize cohort characteristics and demonstrate the breadth and completeness of the data.
  - Cohort characteristics were compared with data on people with mild or moderate HA from the Centers for Disease Control and Prevention (CDC) Community Counts,<sup>3</sup> a public health monitoring program for people with bleeding disorders.
  - For details about the data collection process, please visit our talk on **Monday, December 13, 2021, at 11:45 am in Session 322: A Novel Methodology for Building Longitudinal, Patient-Centric Real-World Datasets in Hemophilia A** (MW Skinner, et al. Abstract #594).



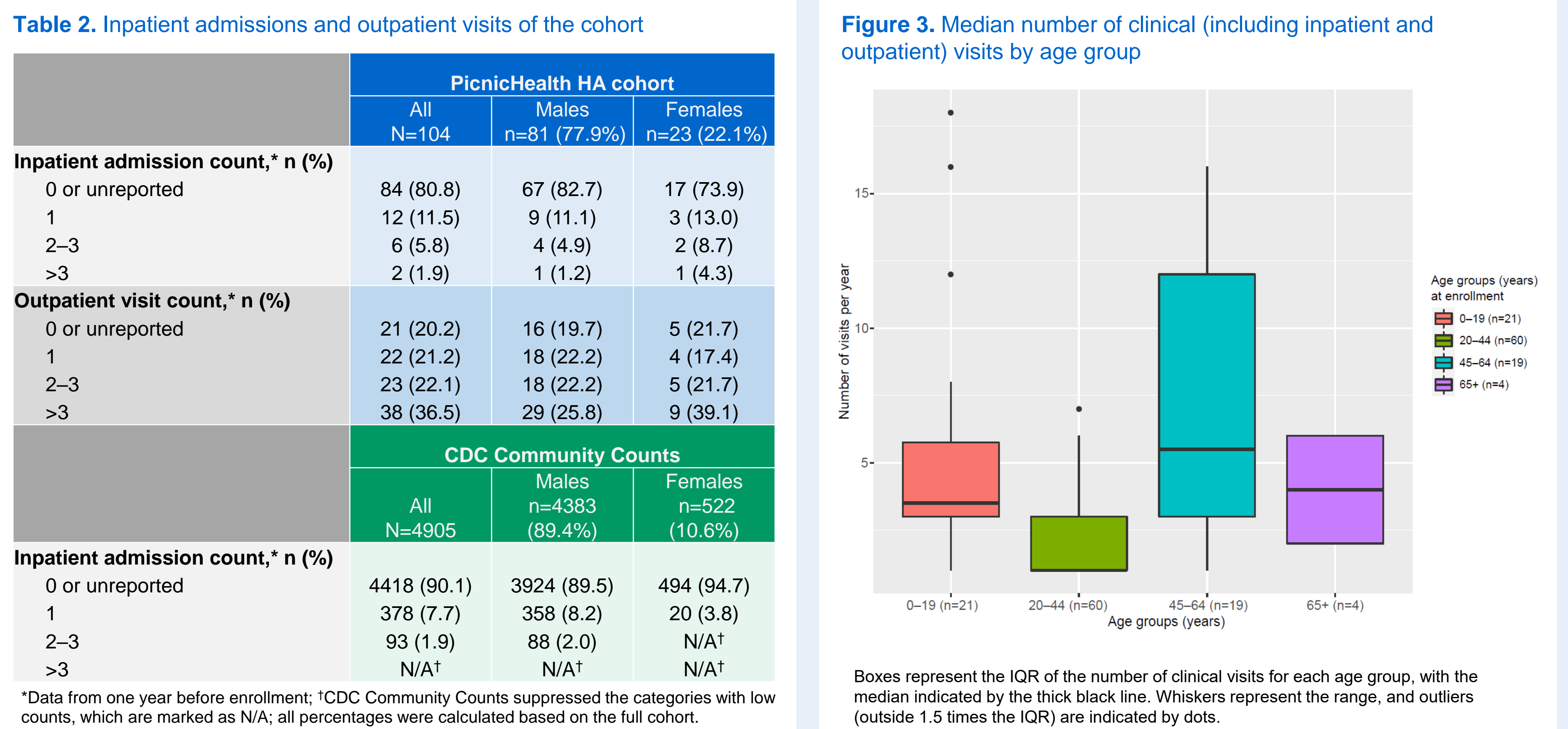
- ### Results: cohort characteristics
- The median (interquartile range [IQR]) age at enrollment was 29 (22–42) years.
  - Age at first diagnosis was available for 55 (52.9%) PwHA; the median (IQR) was 3 (0–13) years
    - Males were diagnosed 10 years younger than females (males, 2 [0–9] years; females, 12 [5–29] years)
    - People with moderate HA were diagnosed at a median (IQR) age of 1 (0–6) years; for people with mild HA, diagnosis was at 5 (0–16) years.
  - With regard to disease severity and ethnicity distributions, the cohort was comparable with the CDC's Community Counts (**Table 1**)
    - However, the present study recruited a higher proportion of female PwHA, which may be due to increasing awareness of PwHA with menstruating potential, and a lower proportion of the pediatric population.

- ### References
- Michele DM, et al. Haemophilia 2014;20:e136–43.
  - Benson G, et al. Blood Transfus 2018;16:535–44.
  - CDC Community Counts data are from the Hemophilia Treatment Center Population Profile Patient Characteristics. Available at: <https://www.cdc.gov/ncbddd/hemophilia/communitycounts/data-community-counts.html>. Accessed November 2021.
- ### Acknowledgments
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- ### Disclosures
- MW:** Current employment with National Hemophilia Foundation, consultancy role at Roche Advisory Panel. **TX, ROA, MA, FS:** current employment with Roche/Genentech and stock or other ownership in Roche. **RHK:** current employment with Roche/Genentech, stock or other ownership in Roche and other privately held companies. **GH, EC:** current employment with PicnicHealth and holder of stock options in PicnicHealth. **MWS:** current employment with Institute for Policy Advancement Ltd; consultancy role at National Hemophilia Foundation, current holder of stocks in IPA Ltd; research funding from BioMarin, Freeline, Roche, Takeda, Unique, honoraria from BioMarin, Takeda, Bayer, Pfizer (DMC), Roche, Sanofi, Spark (DMC); Membership on the Board of Directors or advisory committees of Bayer, ICER, WFH USA, BCBS MAP. **ADS:** current employment at Indiana Hemophilia & Thrombosis Center, Inc; consultancy role at Sangamo Therapeutics, ProMetic Life Sciences and Sigilon; research funding from ProMetic Life Sciences, Sigilon, Bioerativ/Sanofi, Genentech, Kedron Biopharma, Novo Nordisk, Pfizer, Takeda, and Freeline; honoraria from Sigilon, Novo Nordisk, Pfizer, Genentech, and Catalyst Biosciences; membership on the Board of Directors or advisory committees of Sigilon, Novo Nordisk, Genentech, and Sanofi; Speakers Bureau at Novo Nordisk and Genentech.

|  | PicnicHealth HA cohort |                       |                         | CDC Community Counts |                         |                          |
|--|------------------------|-----------------------|-------------------------|----------------------|-------------------------|--------------------------|
|  | All<br>N=104           | Males<br>n=81 (77.9%) | Females<br>n=23 (22.1%) | All<br>N=4905        | Males<br>n=4383 (89.4%) | Females<br>n=522 (10.6%) |
| <b>Severity of hemophilia,* n (%)</b>                        |                        |                       |                         |                      |                         |                          |
| Moderate   | 39 (37.5)              | 39 (48)               | 0 (0)                   | 1825 (37.2)          | 1797 (41)               | 28 (5.4)                 |
| Mild   | 65 (62.5)              | 42 (52)               | 23 (100)                | 3080 (62.8)          | 2586 (59)               | 494 (94.6)               |
| <b>Age group (at enrollment), n (%)</b>                      |                        |                       |                         |                      |                         |                          |
| <2 years   | 0 (0)                  | 0 (0)                 | 0 (0)                   | 220 (4.5)            | 209 (4.8)               | 11 (2.1)                 |
| 2–10 years   | 10 (9.6)               | 7 (8.6)               | 3 (13.0)                | 1045 (21.3)          | 971 (22.2)              | 74 (14.2)                |
| 11–19 years  | 11 (10.6)              | 10 (12.3)             | 1 (4.3)                 | 1109 (22.6)          | 1002 (22.9)             | 107 (20.5)               |
| 20–44 years  | 60 (57.7)              | 46 (56.8)             | 14 (60.9)               | 1425 (29.1)          | 1227 (28.0)             | 198 (37.9)               |
| 45–64 years  | 19 (18.3)              | 14 (17.3)             | 5 (21.7)                | 784 (16.0)           | 682 (15.6)              | 102 (19.5)               |
| 65+ years  | 4 (3.8)                | 4 (4.9)               | 0 (0)                   | 322 (6.6)            | 292 (6.7)               | 30 (5.8)                 |
|  | (n=55)                 | (n=48)                | (n=7)                   |                      |                         |                          |
| <b>Median age at first hemophilia diagnosis (IQR), years</b> | 3 (0–13)               | 2 (0–9)               | 12 (5–29)               | N/A                  | N/A                     | N/A                      |
| <b>Ethnicity, n (%)</b>                                      |                        |                       |                         |                      |                         |                          |
| Hispanic, Latino/a, or Spanish origin                        | 9 (8.7)                | 7 (8.6)               | 2 (8.7)                 | 933 (19.0)           | 843 (19.2)              | 90 (17.2)                |
| Not Hispanic, Latino/a, or Spanish origin, or unknown        | 95 (93.1)              | 74 (91.4)             | 21 (91.3)               | 3972 (81.0)          | 3540 (80.8)             | 432 (82.8)               |
| <b>Race, n (%)</b>   |                        |                       |                         |                      |                         |                          |
| More than one race or unknown                                | 38 (36.5)              | 31 (38.3)             | 7 (30.4)                | 252 (5.1)            | 220 (5.0)               | 32 (6.1)                 |
| White  | 61 (58.7)              | 46 (56.8)             | 15 (65.2)               | 4017 (81.9)          | 3595 (82.0)             | 422 (80.8)               |
| Black or African American                                    | 4 (3.8)                | 3 (3.7)               | 1 (4.3)                 | 449 (9.2)            | 407 (9.3)               | 42 (8.1)                 |
| Other races  | 1 (1.0)                | 1 (1.2)               | 0 (0.0)                 | 187 (3.8)            | 161 (3.7)               | 26 (5.0)                 |
|  | (N=83)                 | (N=64)                | (N=19)                  | (N=2531)             | (N=2201)                | (N=330)                  |
| <b>BMI status (age &gt;19 years),† n (%)</b>                 |                        |                       |                         |                      |                         |                          |
| Underweight (<18.5 kg/m <sup>2</sup> )                       | 2 (2.4)                | 1 (1.6)               | 1 (5.3)                 | 28 (1.1)             | 23 (1.1)                | 5 (1.5)                  |
| Normal (18.5–24.9 kg/m <sup>2</sup> )                        | 13 (15.7)              | 13 (20.3)             | 0 (0)                   | 781 (30.9)           | 670 (30.4)              | 111 (33.6)               |
| Overweight (25.0–29.9 kg/m <sup>2</sup> )                    | 16 (19.3)              | 12 (18.8)             | 4 (21.1)                | 659 (26.0)           | 597 (27.1)              | 62 (18.8)                |
| Obese (≥30.0 kg/m <sup>2</sup> )                             | 28 (33.7)              | 19 (29.7)             | 9 (47.4)                | 877 (34.7)           | 757 (34.4)              | 120 (36.4)               |
| Unreported   | 24 (28.9)              | 19 (29.7)             | 5 (26.3)                | 186 (7.4)            | 154 (7.0)               | 32 (9.7)                 |

CDC Community Counts data are from the Hemophilia Treatment Center Population Profile Patient Characteristics. Accessible at: <https://www.cdc.gov/ncbddd/hemophilia/communitycounts/data-community-counts.html>. \*One symptomatic person with HA in the PicnicHealth cohort had FVIII levels >40%; for the purposes of this study, they are included in the mild HA cohort; †For the PicnicHealth cohort, weight and height information within one year before enrollment was used in the BMI calculation. Percentages were calculated within the age group.

- In the year before enrollment, 83 PwHA had outpatient visits, with a median of three visits for the cohort; 20 PwHA had inpatient admissions (**Table 2**).
- The inpatient visit count was slightly higher in this cohort than CDC Community Counts, where 7.7% had 1 visit over the past year before enrollment, and 1.9% had 2–3 visits. Inpatient admission and outpatient visit frequencies in the year before enrollment were lowest in the 20–44 years age group (median [IQR] 1 [1–3]) (**Figure 3**).



- ## Conclusions
- The patient-centric data collection methods implemented in this study provide a new approach to building cohorts for observational studies for PwHA.
  - PicnicHealth combines routine clinical care data and PROs, and preliminary results suggest congruence when compared with an existing dataset. Participants have access to all records collected, organized in a medical timeline and shareable with care providers, potentially alleviating the burden of care coordination for the patient.
  - The result is a patient-centric approach to data that benefits participants while providing needed data on groups traditionally under-represented in real-world evidence and traditional PwHA cohorts.

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