CAHtalog™
Congenital Adrenal Hyperplasia Patient & Clinical Outcomes in Real-World Practice Settings:

Registry Collecting Longitudinal Data of Patients With Congenital Adrenal Hyperplasia (CAH)

Note: We refer to classic CAH as CAH; deviations from classic CAH are denoted by using specific terminology (e.g., non-classic CAH).

NEUROCRINE®
BIOSCIENCES

CAHtalog™ Registry

CAHtalog™

Congenital Adrenal Hyperplasia
Patient & Clinical Outcomes in Real-World Practice Settings

The CAHtalog registry: A community-driven research opportunity

What is the CAHtalog registry?
CAHtalog is a patient registry, or collection of patient clinical data, for classic CAH patients
Its mission is to advance clinical research and develop new treatments for CAH

PicnicHealth

PicnicHealth is our technology partner to generate large-scale registry data from patients' medical records

NEUROCRINE BIOSCIENCES

As the sponsor, Neurocrine leads the CAHtalog study design and analysis, and provides funding for the registry

cares

CARES drives recruitment and ensures that CAHtalog's research serves the needs of the CAH patient community

CAH Researchers

*CAH researchers will analyze CAHtalog data to advance clinical research and develop new treatments*

Patients with Classic CAH

Patients have the opportunity to directly contribute to research by signing up and sharing their experience living with CAH

PicnicHealth. Congenital adrenal hyperplasia (CAH) registry. Accessed May 20, 2024. https://picnichealth.com/cah.

# CAHtalog leverages advanced AI technology to collect and analyze medical records from patients living with CAH

To enroll as a patient or caregiver with classic CAH, scan QR or visit https://picnichealth.link/cah

Consent & Enroll in 10min!
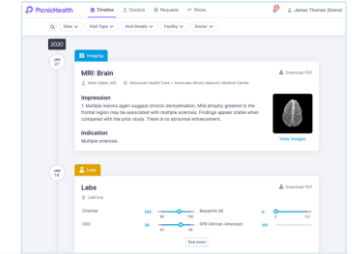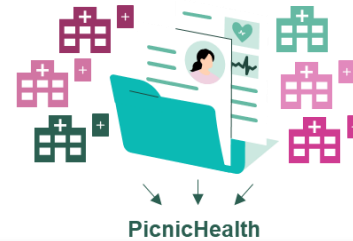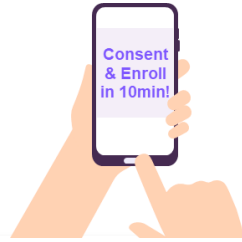
After enrolling, we will ask for the **contact information of their most recent primary care provider and endocrinologist.** Nothing else is required

PicnicHealth

PicnicHealth collects medical records from all available healthcare providers for the last 5+ years. There is no need to fill out forms or call hospitals

Patients will receive a copy of their digitized medical records in a **well-organized, patient-friendly "PicnicHealth Timeline"**

Take a short survey & contribute to CAH research
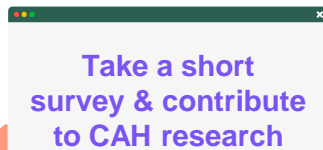
Patients may participate in **optional** __bi-annual surveys__ to further share their experience with CAH

Vitals

Medications

Diagnoses

Lab Results

Procedures

Thousands of datapoints are **abstracted from the patient's medical records**

Qualified researchers can apply to **analyze *de-identified* CAHtalog data** to advance CAH research and develop new treatments

AI, artificial intelligence.

PicnicHealth. Congenital adrenal hyperplasia (CAH) registry. Accessed May 20, 2024. https://picnichealth.com/cah.

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Why is CAHtalog important?

Medical records form a foundation of clinical research, and answer key questions such as:
- Natural history of CAH
- Real-world burden of illness
- Glucocorticoid dose vs. clinical outcomes
- How routine care in real-world differs from clinical guidelines
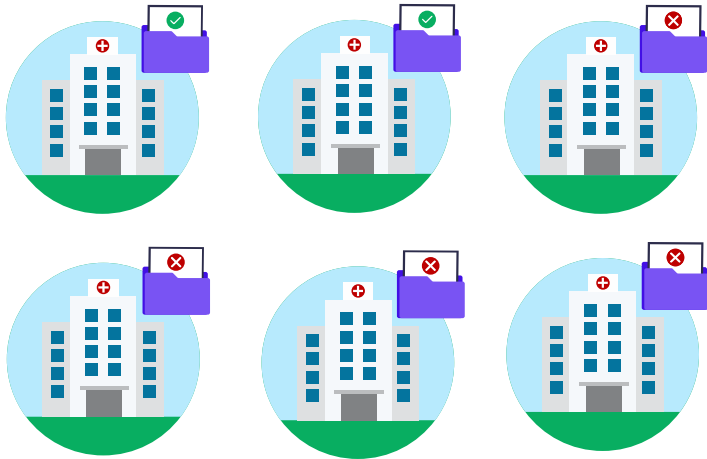

Advancing clinical research can lead to:
- Improved clinical guidelines
- New treatments for CAH
- Clinicians and policymakers understanding the importance of CAH

Because CAH is rare, it is very challenging for CAH researchers to access real-world data with a sufficient sample size and generalizability. Thus, there are still major gaps in our understanding of CAH.

PicnicHealth. Congenital adrenal hyperplasia (CAH) registry. Accessed May 20, 2024. https://picnichealth.com/cah.

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Challenges in Accessing Complete Medical Records

MyChart and other local portals typically will not have records from every hospital/clinic the patient has visited

Images from CT's and MRI's are often unavailable in MyChart and instead must be shared via CD's and DVD's
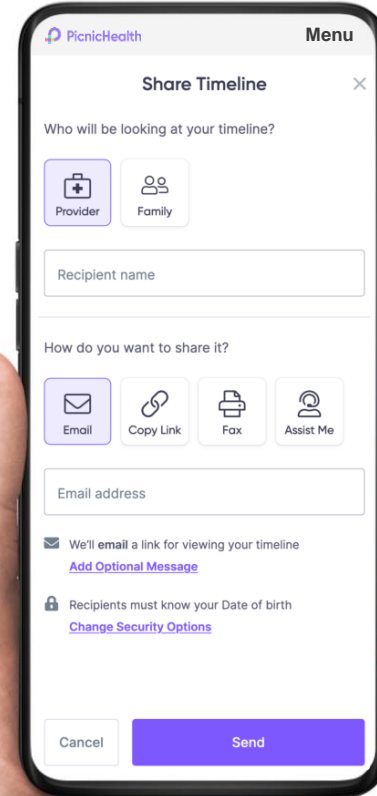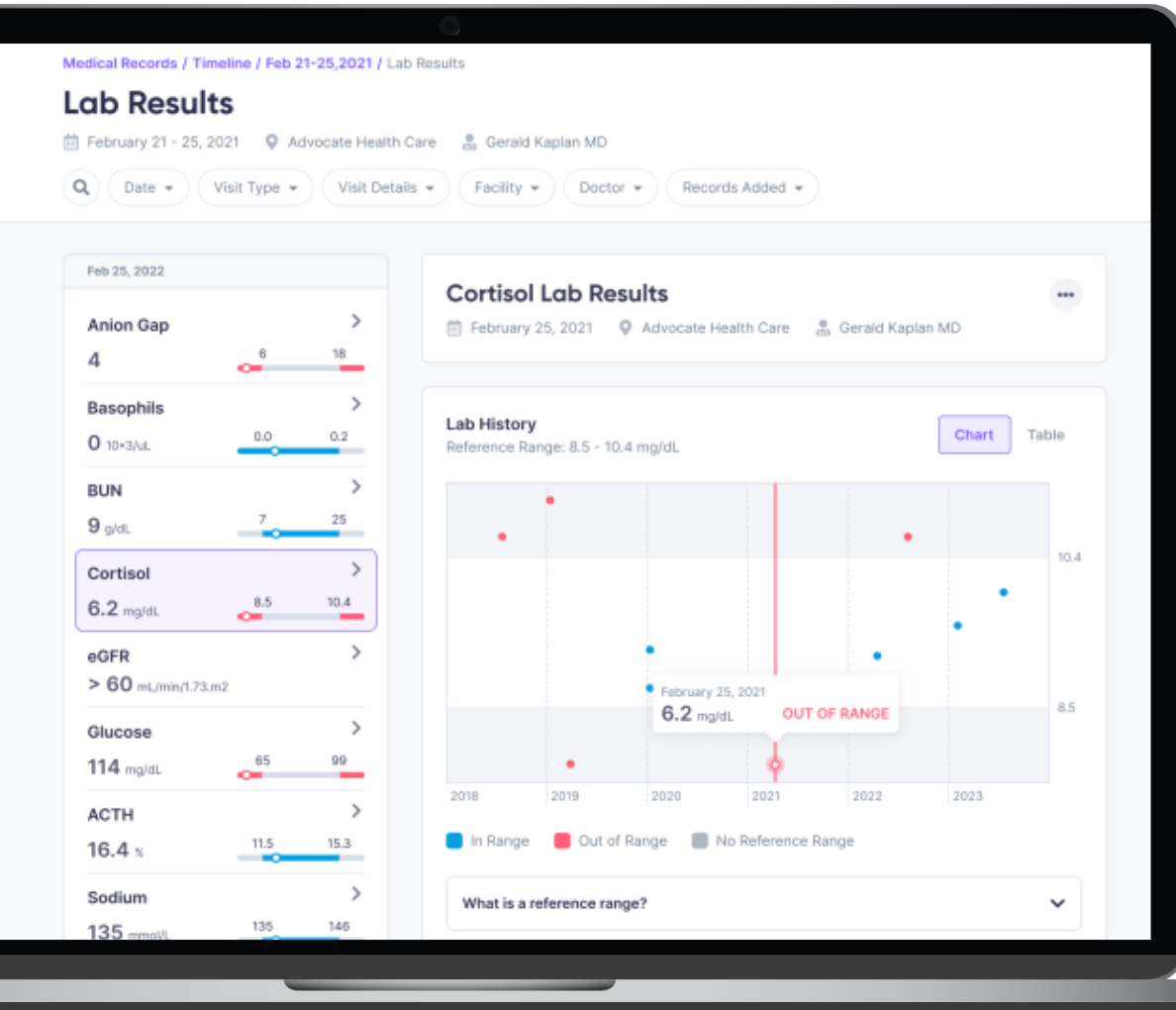

EMERGENCY ROOM

When patients seek emergency care while traveling out of town, the **ER clinician may be unable to quickly access important medical records**

CT, computed tomography; ER, emergency room; MRI, magnetic resonance imaging.

The PicnicHealth timeline provides a comprehensive solution to the challenges of accessing complete medical records

Click here for demo:

https://demo.picnichealth.com/records

This example PicnicHealth Timeline is for demonstration purposes only

CAHtalog Registry Summary

What is it?	CAHtalog is a **patient registry,** or collection of patient clinical data, for CAH patients
What is its purpose?	Its mission is to advance clinical research and develop new treatments for CAH by obtaining information about how patients with CAH are managed by their healthcare providers in a **real-world setting**
How does it work?	Adults and children living with CAH will consent to have PicnicHealth collect their medical records, which will be **stripped of any personally identifiable information** and combined into a de-identified CAH database
Who supports it?	CAHtalog is sponsored by **Neurocrine Biosciences, Inc**., in partner with and supported by **CARES Foundation**, and operationalized by **PicnicHealth**

Click here for information on CAHtalog data

PicnicHealth. Congenital adrenal hyperplasia (CAH) registry. Accessed May 20, 2024. https://picnichealth.com/cah.

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Neurocrine Medical Affairs

www.neurocrinemedical.com

📞 1-877-641-3461

Appendix

CAHtalog™ Registry Data

White P et al. Poster presented at PES; May 2-5, 2024, Chicago IL.

Lekarev O et al. Poster presented at PES; May 2-5, 2024; Chicago, IL.

Pediatric patients had early growth acceleration followed by blunted pubertal growth

Girls, 2 to 17 years
n=23

Boys, 2 to 17 years
n=19
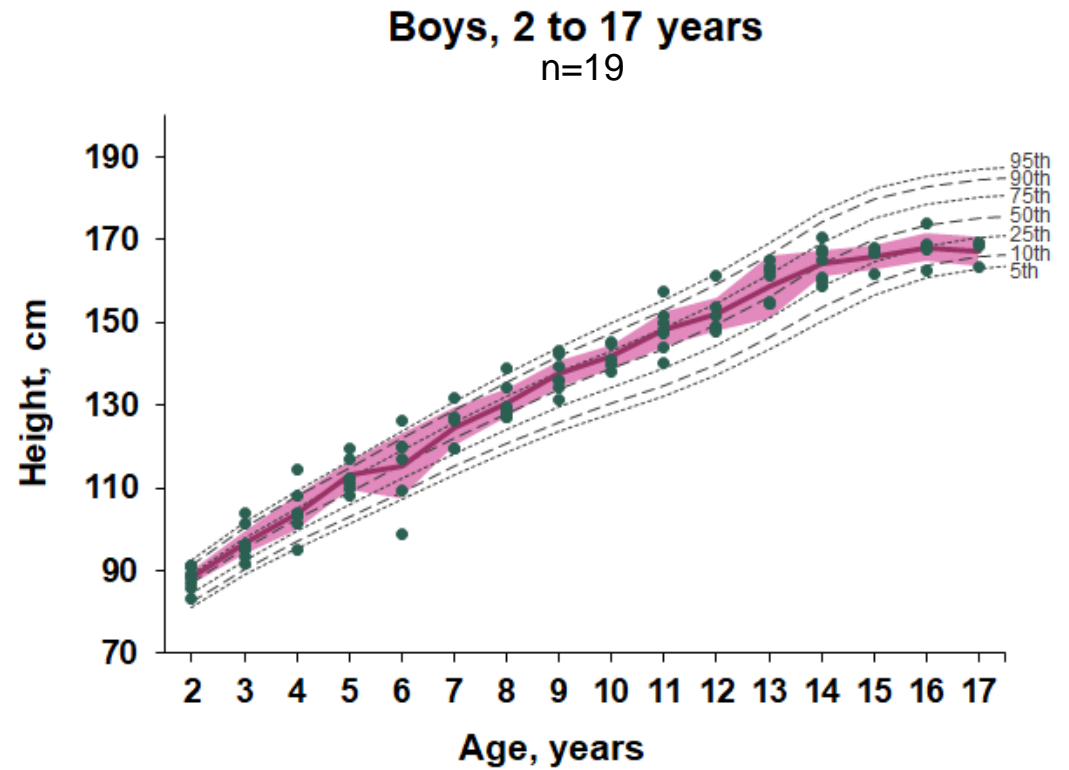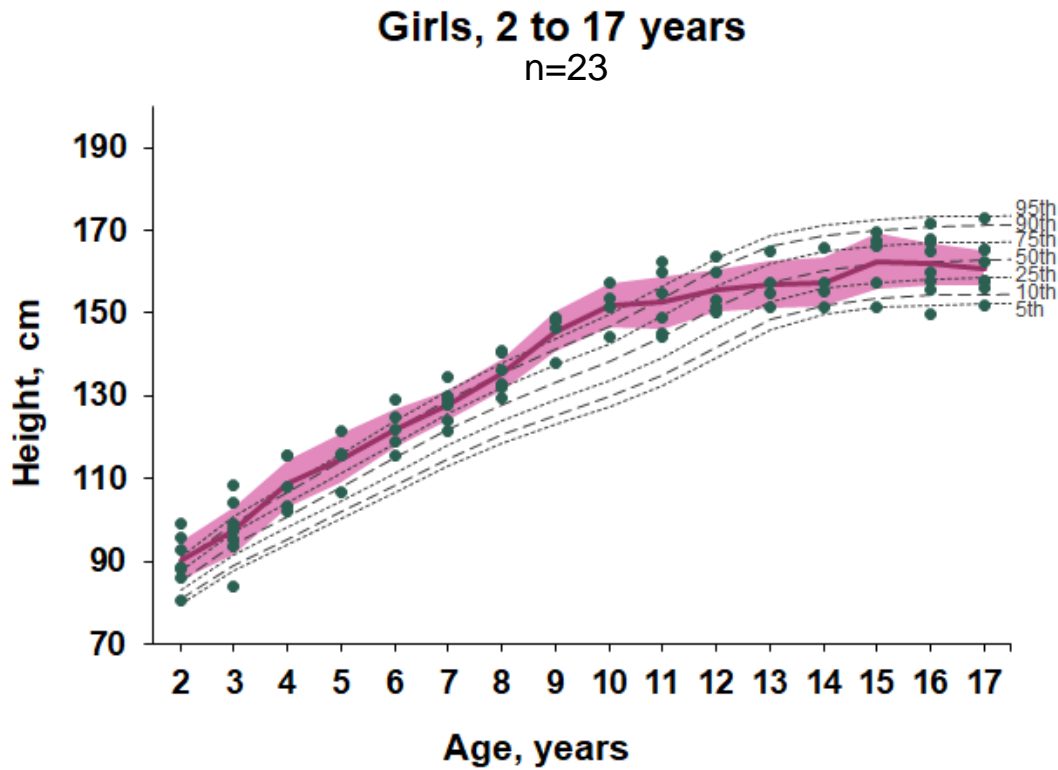
Key Results

Pediatric patients had early growth acceleration followed by blunted pubertal growth. This trend was more pronounced in females, with mean height-for-age generally exceeding the 90th percentile in ages 4-10 but dropping below the 50th percentile at ages 13-17

White P et al. Poster presented at PES; May 2-5, 2024, Chicago IL.

Obesity was observed from childhood through adulthood

Girls, 2 to 17 years
n=23

Boys, 2 to 17 years
n=19
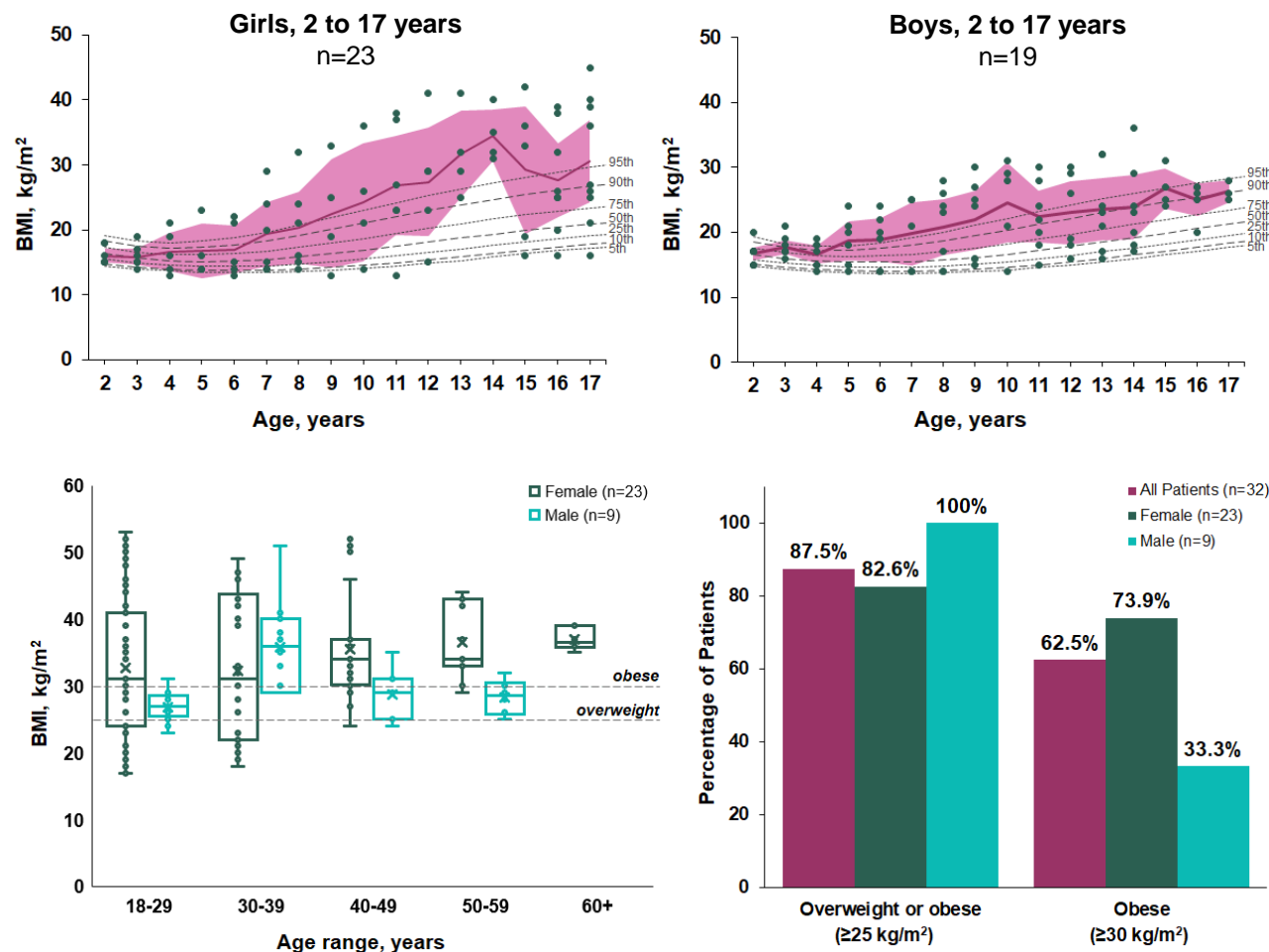
Obesity was observed from childhood through adulthood. In adults, obesity (BMI ≥30) was highly prevalent (64%), exceeding NHANES 2017-2018 general population prevalence (42%)

BMI, body mass index; NHANES, National Health and Nutrition Examination Survey.

White P et al. Poster presented at PES; May 2-5, 2024, Chicago IL.

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We aimed to visualize the transition of patients through health states based on lower vs. higher GC doses and A4 levels

	Lower GC dose	Higher GC dose
Higher A4	**PED**: ≤11 mg/m2/d HCe, A4 ≥ULN **ADULT**: ≤20 mg/d HCe, A4 ≥ULN	**PED**: >11 mg/m2/d HCe, A4 ≥ULN **ADULT**: >20 mg/d HCe, A4 ≥ULN
Lower A4	**PED**: ≤11 mg/m2/d HCe, A4 <ULN **ADULT**: ≤20 mg/d HCe, A4 <ULN	**PED**: >11 mg/m2/d HCe, A4 <ULN **ADULT**: >20 mg/d HCe, A4 <ULN

A4, androstenedione; GC, glucocorticoid; HCe, hydrocortisone equivalent; ULN, upper limit of normal.

The vast majority of patients (97%) had higher GC's and/or loss of androstenedione control at some point, or multiple points, of their journeys

A4, androstenedione; GC, glucocorticoid; HCe, hydrocortisone equivalent; ULN, upper limit of normal.

Lekarev O et al. Poster presented at PES; May 2-5, 2024; Chicago, IL.

1st Recorded Data

- Higher GC + uncontrolled androgens
- Higher GC + controlled androgens
- Lower GC + uncontrolled androgens
- Lower GC + controlled androgens

Last Available Data

	Lower GC dose	Higher GC dose
Higher A4	PED: ≤11 mg/m2/d HCe, A4 ≥ULN ADULT: ≤20 mg/d HCe, A4 ≥ULN	PED: >11 mg/m2/d HCe, A4 ≥ULN ADULT: >20 mg/d HCe, A4 ≥ULN
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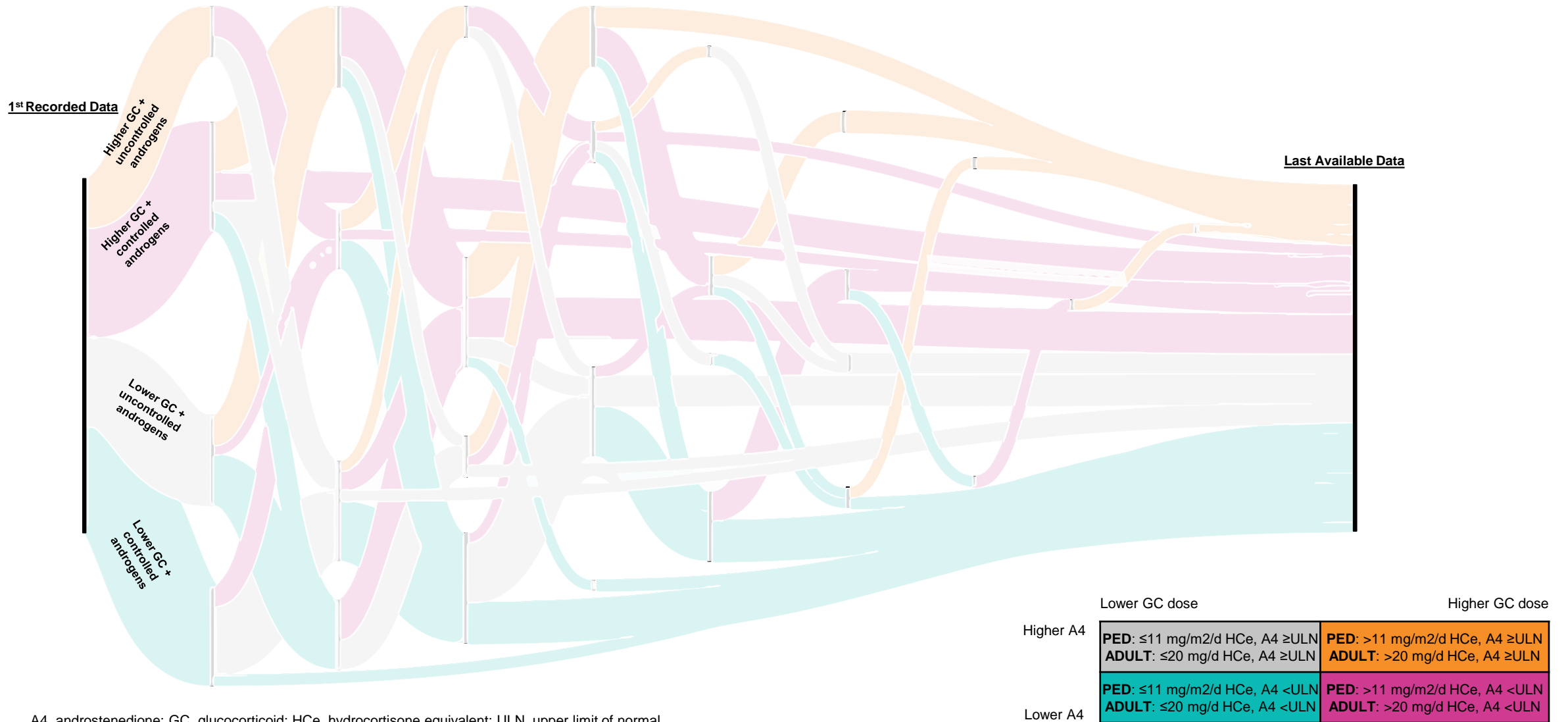Lekarev O et al. Poster presented at PES; May 2-5, 2024; Chicago, IL.

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Lower GC + controlled androgens

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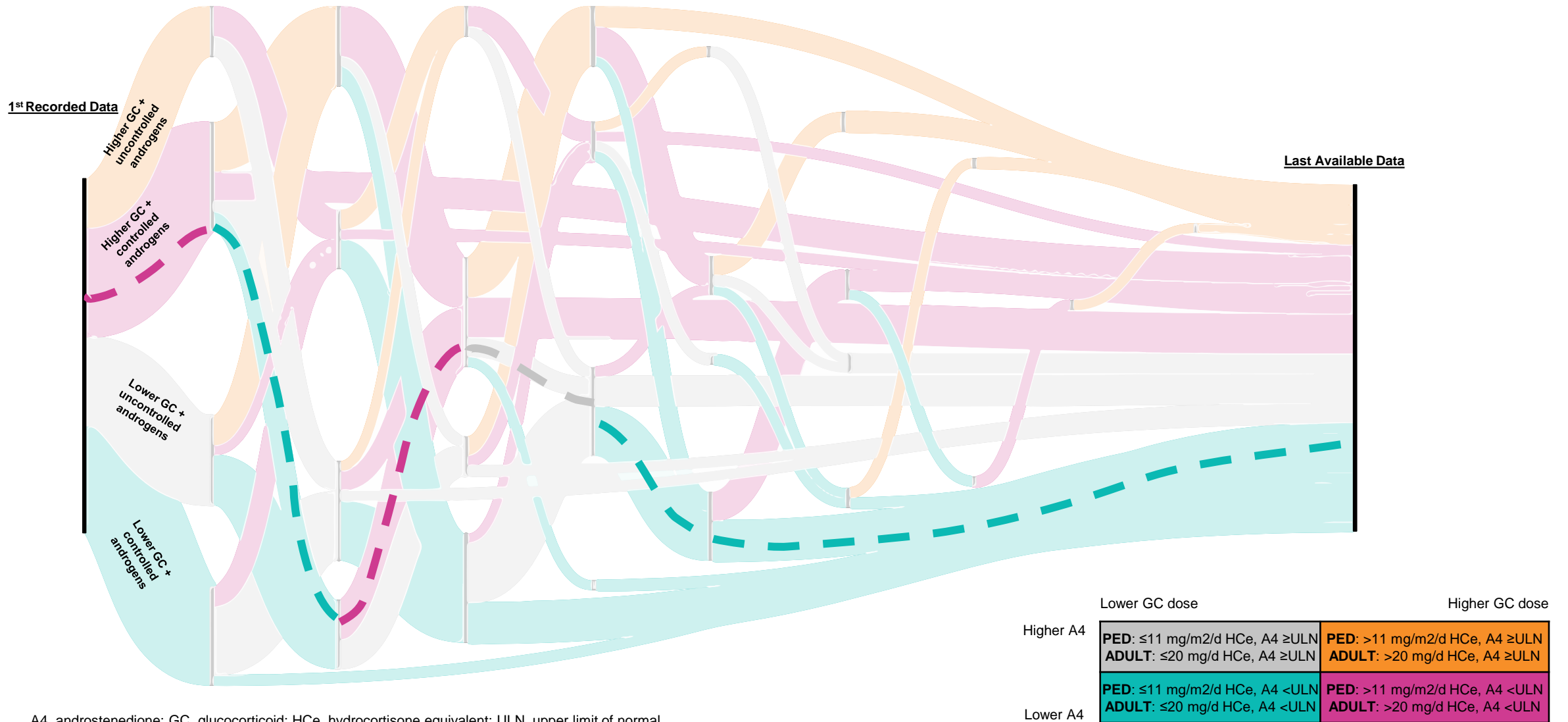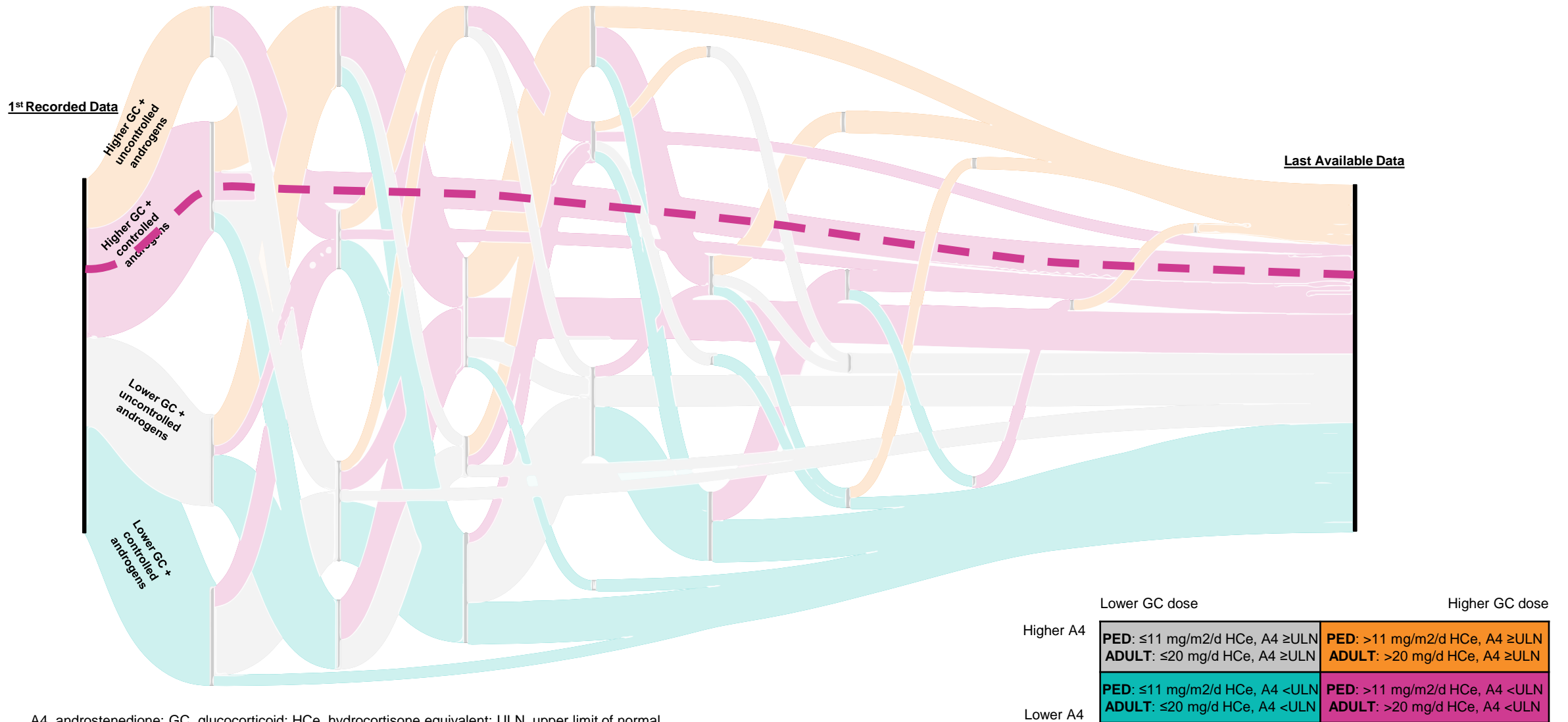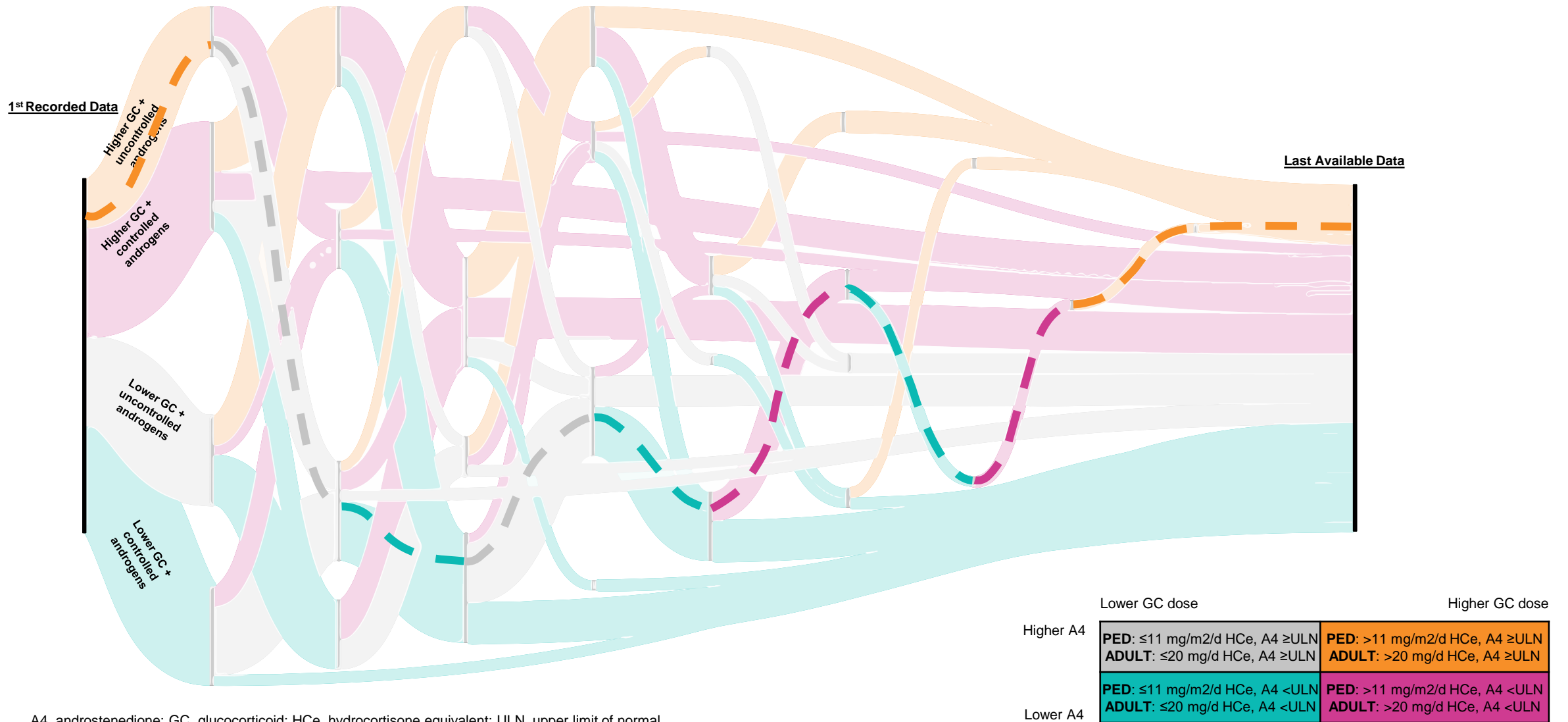Lekarev O et al. Poster presented at PES; May 2-5, 2024; Chicago, IL.

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Higher GC + controlled androgens

Lower GC + uncontrolled androgens

Lower GC + controlled androgens

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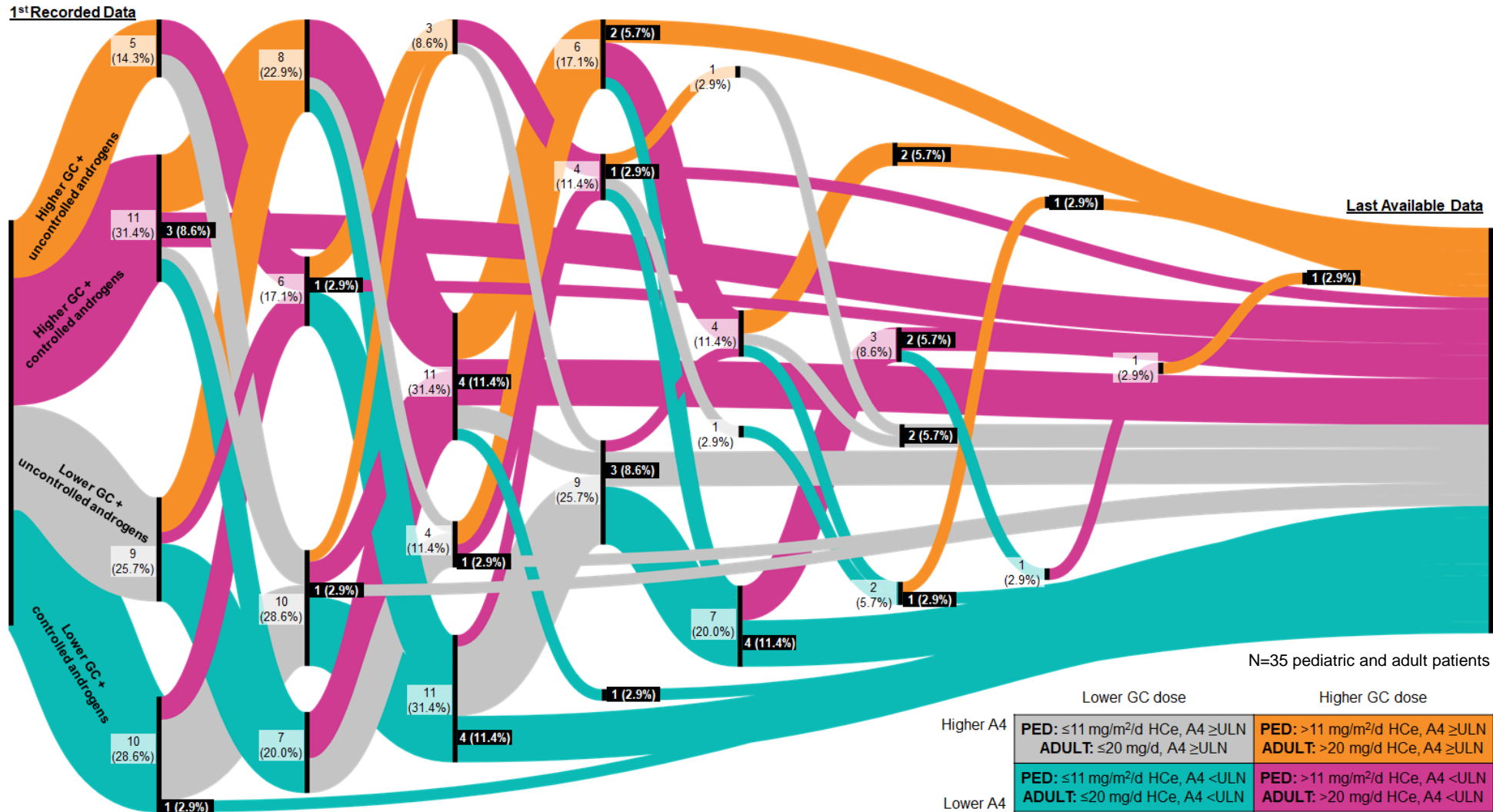Lekarev O et al. Poster presented at PES; May 2-5, 2024; Chicago, IL.

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Key Takeaway Taken together, these analyses underscore that **with existing treatment approaches, disease control today does not equate to disease control tomorrow**

Important considerations for interpreting Sankey diagrams of changes in health states include:

- **Absence of Temporal Context:** Diagram depicts order of transitions between health states for each patient, but doesn't show length of time patients spend in each health state

- **Observation Period:** Each patient journey represented equally regardless of each patient's observation period, with interquartile ranges of 3 to 8 years

- **Number of Records per Patient:** The number of available matched A4-GC dose records varied for each patient, ranging from 3 to 20 records

- **Timing of Androstenedione Levels**: The captured androstenedione levels might not fully characterize disease control due to lack of information about the timing of the measurement relative to GC dose

- **Sample Bias:** As is typical of many registry studies, patients who voluntarily enrolled into CAHtalog may have been more likely to be engaged in their care
  - **70% received care at a CoE or large academic hospitals**
  - *Thus, these data likely underestimate the true burden of classic CAH*

A4, androstenedione; CoE, Center of Excellence; GC, glucocorticoid.