CAHtalog™
Congenital Adrenal Hyperplasia Patient & Clinical Outcomes in Real-World Practice Settings:
Registry Collecting Longitudinal Data of Patients With Congenital Adrenal Hyperplasia (CAH)

NEUROCRINE® BIOSCIENCES

CAHtalog^{TM}: Objective

> **Obtain information about how adults and children with CAH are managed by their healthcare providers in a real-world setting**

Key objectives are to describe:

- Demographic, clinical, and socioeconomic characteristics in people with CAH

- Treatment patterns and standard disease management in people with CAH and observe treatment related and disease-related features

- Clinical, economic, and patient/caregiver reported outcomes of treatment approaches for people with CAH in the context of real-world medical practice

- The natural progression (longitudinally) of the disease in the current standard of care framework

Goals:

- Support research in the entire CAH community

- Determine and report the common clinical characteristics of a representative CAH population

Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021.

Registry Definition

Registry Definition

- A patient registry is an organized system that uses **observational** study methods to **collect data** in a systematic and comprehensive way[1]

- The data extracted from the registry (clinical and other) can be utilized to[1]:

  - Evaluate specified outcomes for a population defined by a **particular disease**, condition, or exposure

- Registries are classified according to how their populations are defined[1]

  - Examples[1]:

    - Product registries include patients who have been exposed to biopharmaceutical products or medical devices

    - Health services registries consist of patients who have had a common procedure, clinical encounter, or hospitalization

    - **Disease or condition registries are defined by patients having the same diagnosis, such as cystic fibrosis or heart failure**

CAHtalog is a disease registry[2]

1. Gliklich RE, Leavy MB, Dreyer NA (sr eds). Registries for Evaluating Patient Outcomes: A User's Guide. 4th ed. (Prepared by L&M Policy Research, LLC, under Contract No. 290-2014-00004-C with partners OM1 and IQVIA) AHRQ Publication No. 19(20)- EHC020. Rockville, MD: Agency for Healthcare Research and Quality; September 2020. Posted final reports are located on the Effective Health Care Program search page. DOI: https://doi.org/10.23970/AHRQEPCREGISTRIES4. 2. Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021.

CAHtalog™ Registry

CAHtalog™ Registry

CAHtalog is an IRB approved registry designed to collect medical history information about people diagnosed with CAH retrospectively and prospectively

- People living with classic CAH can consent to having their medical records, which will be stripped of any personally identifiable information, combined into a de-identified CAH database

- This registry will enable participants and their families to easily share their real-world data for the advancement of CAH research, such as:
  - Management
  - Natural history
  - Treatment patterns

- CAHtalog is a direct to patient, decentralized registry

- The CAHtalog registry is sponsored by Neurocrine Biosciences, Inc., operationalized by PicnicHealth, and supported by CARES Foundation

Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021.

Key Roles

CAHtalog Registry is sponsored by Neurocrine Biosciences, Inc.[1]

CARES Foundation[1,2]**:**
- Patient advocacy foundation that leads in the effort to improve the lives of the CAH community and seeks to advance quality health care through advocacy, education, research, and support
- Endorses CAHtalog and has helped in the planning and implementation of the registry

PicnicHealth's research platform will collect medical records, remove personally identifiable information, and structure the data by querying medical records using machine learning capability[1]
- Will not share personal information with CARES or Neurocrine Biosciences, Inc.[1]

1. Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021.

2. CARES Foundation. Our Mission - CARES Foundation. [online] Available at: https://caresfoundation.org/our-mission/. Accessed January 7, 2022.
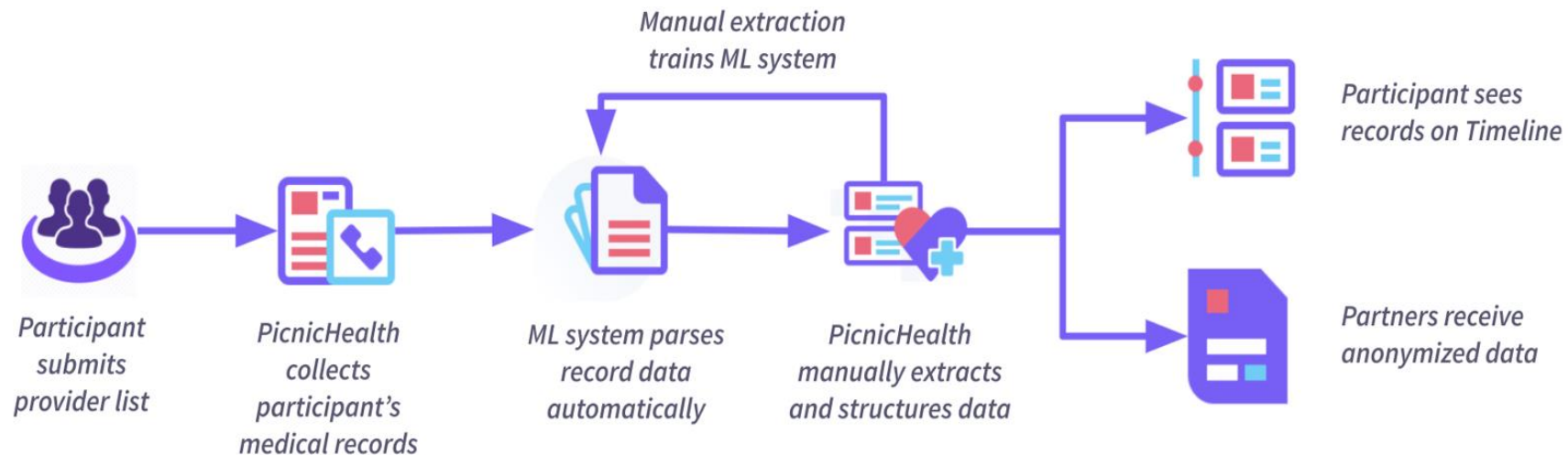
CAHtalog™: Enrollment

- Enrollment:
  - People diagnosed with classic CAH who live in the U.S. are eligible to participate, including both adults and children with classic CAH
  - There is no upper or lower age cut off for enrollment
  - Caregivers can enroll for pediatric participants
  - Sign up takes 5 – 10 minutes through the CAHtalog website[a]
  - There is no cost associated with participation in CAHtalog
- Participant benefits:
  - Contribute to CAH research and share their unique patient journey and voice through optional patient reported outcomes (PROs), without the need for in-person visits
  - Receive access to fully digitized medical records via their own PicnicHealth Timeline, which allows participants to view, download, and share their medical histories
  - Receive future updates on findings/publications
- Provider role:
  - No active work (data entry) required
- All data shared with researchers is anonymized

[a]Enrollment requires the patient's last 4 digits of their SSN and the names of their physicians. Only 1 consent is needed by the patent or caregiver, without any additional action needed thereafter.
Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021.

Content is intended for healthcare provider educational purposes only.

CAHtalog™: Data Collection and Extraction

- Information is collected passively and does not require additional data entry by a provider[1]
- PicnicHealth gathers imaging files, test results, doctors' notes, etc. from all types of US providers and then removes any identifying information[1]
  - Optional patient reported outcomes (PROs) can be completed through the website
- The data extraction process leverages both machine learning predictions and trained human reviewers[2]:

Manual extraction trains ML system

Participant submits provider list → PicnicHealth collects participant's medical records → ML system parses record data automatically → PicnicHealth manually extracts and structures data → Participant sees records on Timeline / Partners receive anonymized data

ML, machine-learning. Figure adapted from How PicnicHealth Generates Real-World Data from Medical Records[2].

1. Congenital Adrenal Hyperplasia (CAH) Registry. PicnicHealth. https://picnichealth.com/cah Accessed December 21, 2021. 2. How PicnicHealth Generates Real-World Data from Medical Records. PicnicHealth. https://assets.website-files.com/6074d822c3c38b07ef63efe5/609d684878e516dd99c3a4ae_How%20PH%20Generates%20RWD%20from%20Medical%20Records-compressed.pdf Accessed January 11, 2021.

CAHtalog™: Extracting data for Research

Researchers need data from actual people with CAH in order to identify patterns and fill knowledge gaps

- Medical records can provide a **real-world perspective** on how participants experience CAH (i.e., symptoms, health status, treatments)

- Once the participant's medical history is made anonymous, it will be compiled with medical history information from other people with CAH in a database to create a **resource for researchers**

- Information extracted from this registry can help the CAH community gain a better understanding of the disease, such as:
  - Management
  - Natural history
  - Treatment patterns
  - Patient reported or observer-reported outcomes

- The registry can raise awareness of CAH, which may help improve the care of future people living with CAH

- CAHtalog is an open access registry available to all researchers[a]

[a]Application accepted and reviewed though the Data Governance and Scientific Review Committee. The committee comprises of key academic opinion leaders in the field.
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CAHtalog™: The PicnicHealth Timeline

Each participant who signs up will receive their own PicnicHealth Timeline to view, manage, and share their longitudinal health histories
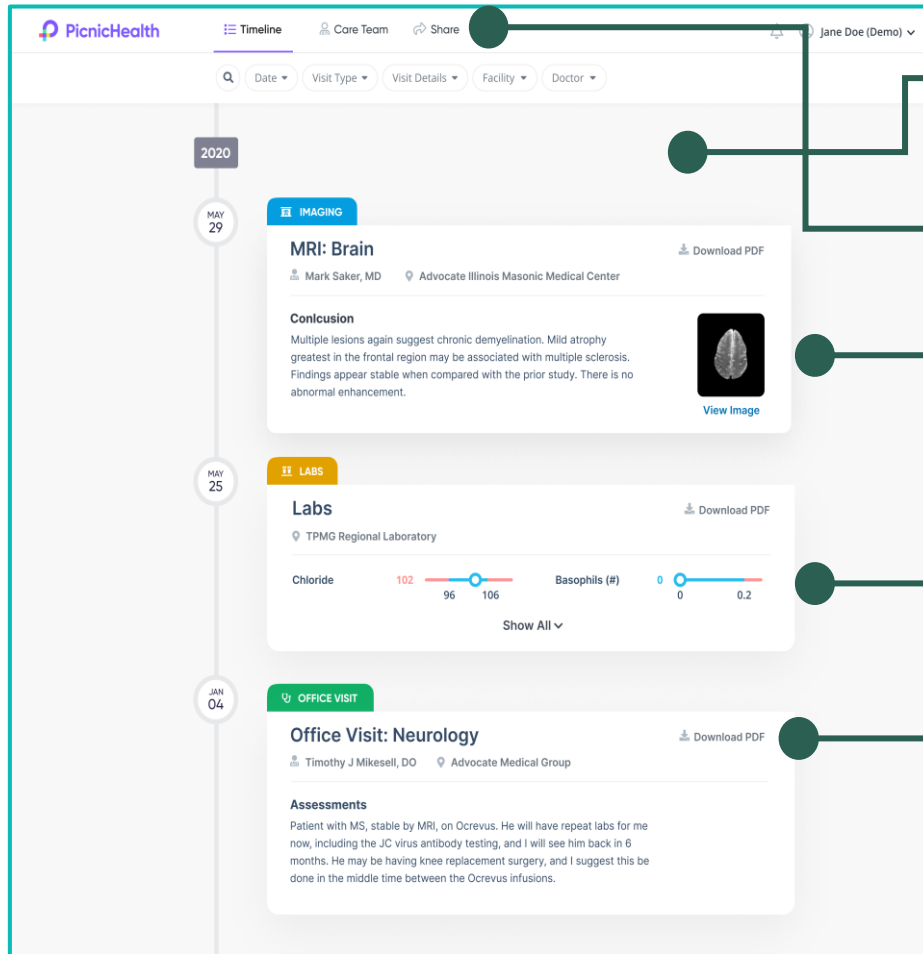
Access all medical records in one place

Share records with family and physicians

View images through our FDA approved & HIPAA compliant image viewer

Track all labs longitudinally

Download original source records

This example PicnicHealth Timeline is for demonstration purposes only.

Demo PicnicHealth Timeline. PicnicHealth. https://demo.picnichealth.com. Accessed January 12, 2022.

CAHtalog™: Summary

What is it?	An IRB approved, direct-to-patient registry, used to capture a **real-world sample** of patient care characteristics
What is its purpose?	Obtain information about how adults and children with CAH are managed by their healthcare providers in a **real-world setting**
How does it work?	Adults and children living with classic 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**

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cares FOUNDATION
CONGENITAL ADRENAL HYPERPLASIA

PicnicHealth

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