



NAAF Webinar  
Series:  
Patient Guide to  
Clinical Trials and  
Drug Development:  
Phases and  
Terminology  
Explained

Presented by:

Danielle Quarles, MPH

Senior Director, Clinical Operations

Sana Biotechnology, Inc.

Wednesday, 15-Feb-2023

# My Background



- I am a mom to two awesome alopecians- Connolly and Damon
  - Connolly is now 14, and was diagnosed with AA at age 3
  - Damon is now 8, and was diagnosed with AA at age 18 months
- I have 20 years of clinical operations and clinical program management experience
  - I serve as the operational strategy, direction and oversight of clinical programs at a biotech company
  - In developing clinical trial protocols, I am the voice of the site and the patient
  - In 2022 I spoke at the ASGCT Annual Conference on "Diversity in Research"
  - In 2018 I was named PharmaTimes Clinical Researcher of the Year (CROY) Gold Medal Winner
- I have a Master of Public Health (MPH) degree from Johns Hopkins

What are clinical trials? Why do we need them?

Clinical trials are research studies performed in people that are aimed at evaluating a medical, surgical or behavioral intervention.

They are the primary way that researchers find out if a new treatment is



SAFE



EFFECTIVE

# What makes a clinical trial reliable?

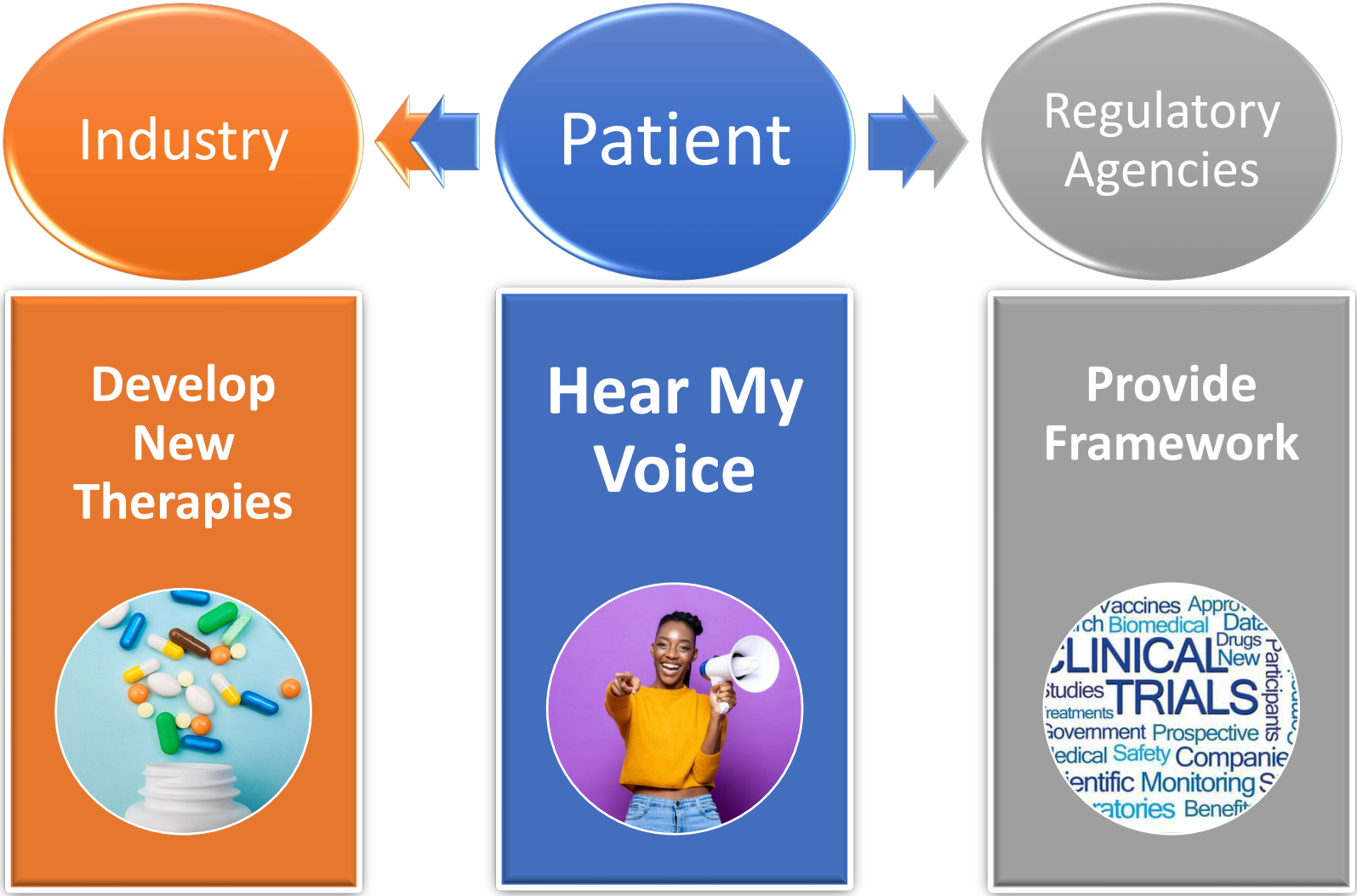
- Rigorous science
- Reproducibility
- Regulation
  - Federal law (eg, FDA)
  - International guidance

Two of the cornerstones of science advancement are **rigor** in designing and performing scientific research and the **ability to reproduce** biomedical research findings. The application of rigor ensures robust and unbiased experimental design, methodology, analysis, interpretation, and reporting of results. When a result can be reproduced by multiple scientists, it validates the original results and readiness to progress to the next phase of research. This is especially important for clinical trials in humans, which are built on studies that have demonstrated a particular effect or outcome.

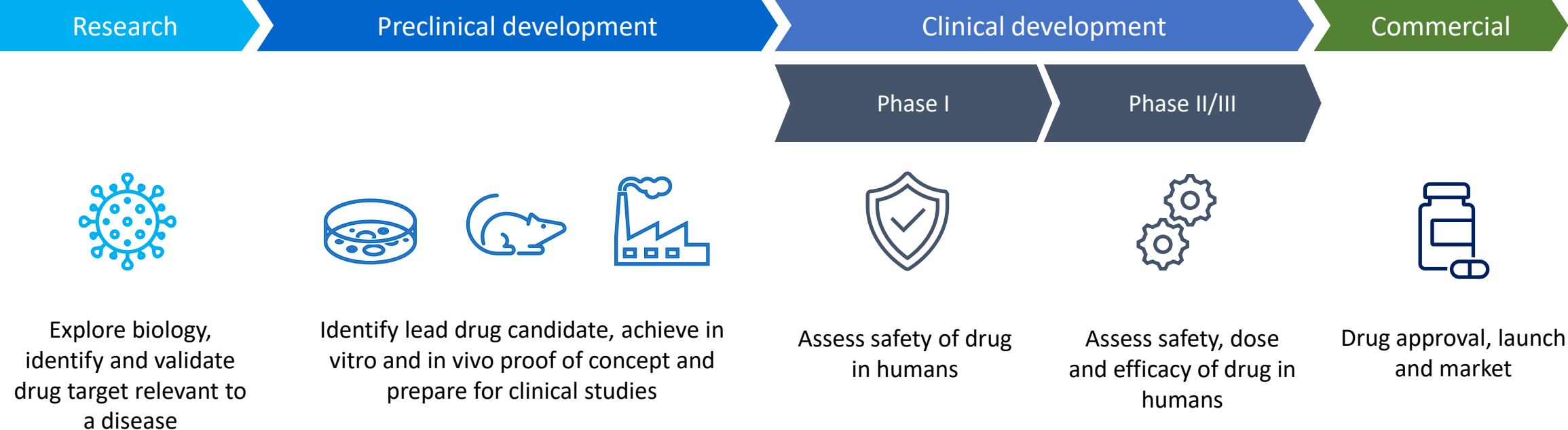
—Francis S. Collins, MD, PhD, NIH  
Director

# What role do patients play in drug development?

## Voice of the Patient in Developing New Therapeutics



# Drug development is a complex and dynamic process



# What are clinical trial phases?

IND  
Submission

Phase 1 – 3 Clinical Trials

Marketing  
Application,  
Review &  
Approval

Product  
Launch

Post-Marketing  
Surveillance

## Phase 1

### Human Pharmacology/ First in Human (FIH)

- Purpose:
  - Safety and tolerability
  - Pharmacokinetics (ADME)
  - Pharmacodynamics
- Dosing:
  - Dose finding/ranging, often starting at a sub-therapeutic dose
  - Open-label
- Study Length: Several months
- Study sites: 1-10 clinical sites
- Participants: 20-100 healthy volunteers, or people with the disease/condition (where necessary, eg oncology)

**~70% of drugs move onto the next phase**



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Phase 2

## Therapeutic Exploratory

- Purpose:
  - Safety and tolerability
  - Efficacy
  - Proof of Concept (POC)
- Dosing:
  - Typically open-label
  - Can be blinded, comparative
- Study Length: Several months to 2 years
- Study sites: 5-50 clinical sites
- Participants: 100-500 people with the disease/condition

**~33% of drugs move onto the next phase**



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Phase 3

## Therapeutic Confirmatory

- Purpose:
  - Efficacy
  - Monitoring for adverse reactions
- Dosing:
  - Blinded
  - Comparative
- Study Length: 1-4 years
- Study sites: >50 clinical sites
- Participants: 300-3,000 people with the disease/condition

**~25-30% of drugs move onto the next phase**

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### Phase 4

#### Post Marketing Surveillance

- Purpose:
  - Safety surveillance (pharmacovigilance)
  - Real World Evidence (RWE) and outcomes data gathering
- Dosing: Therapeutic dosing
- Study Length:
  - Several years
  - Dependent upon regulatory commitments
- Study sites: Hundreds – thousands of sites
- Participants: Several thousand people with the disease/ condition

For every 100 drugs entered into Phase 1 trials, fewer than 10 will receive regulatory approval

# How is the safety of clinical trial participants protected?

Researchers follow a strict set of rules to ensure that participants are safe. The rules are enforced by the US federal government via the FDA.

## Study Protocol

- Each clinical trial follows a carefully crafted study plan called a protocol. This describes what the researchers will do
- The principal investigator is responsible for ensuring that the protocol is followed

## Institutional Review Board (IRB)

- An IRB at each site must approve every clinical trial in the United States
- IRB is made up of doctors, scientists and lay people
- The IRB regularly reviews the study and its results
- The IRB ensures that risks (or potential harm) to participants are as low as possible

## Data and Safety Monitoring Committee

- Some (but not all) clinical trials are supervised by this committee
- The committee is made up of experts in your condition who periodically review results as the study progresses
- If they find that the experimental treatment is not working or is harming patients, they will stop the trial immediately

# Clinical Trial Roles and Responsibilities

## Sponsor (eg Pharma/ Biotech)

- Design medically and scientifically sound clinical protocols
- Provide medical oversight and expertise
- Monitor safety

## Patient

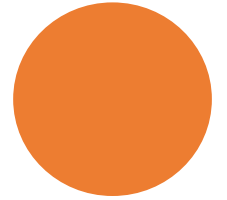
- Understand risk
- Participate in the informed consent process
- Participate in clinical trial assessments

## Investigator

- Medical care of patients
- Ensure compliance with clinical protocol

## Investigational Review Board (IRB)

- Safeguards the rights, safety, and well-being of trial participants



Participating in a clinical trial may have risks, but it may also have benefits

## Potential Benefits

You may receive a new treatment before it is available widely

You play an active role in driving your healthcare

Researchers may provide you with medical care as a part of your treatment

You may have the chance to help others get a better treatment for their health concerns in the future

## Potential Risks

The new treatment may cause serious side effects or be uncomfortable

The new treatment may not work, or it may not be better than what is already available

You may NOT be a part of the experimental group, and you may not receive the new treatment. This means that you might receive a placebo or standard of care treatment

The clinical trial may be an inconvenience to you. Required appointments and procedures may take a lot of time, or require you to travel to the investigator site many times



**ARE YOU THE  
PLACEBO?**

**AREN'T YOU?**

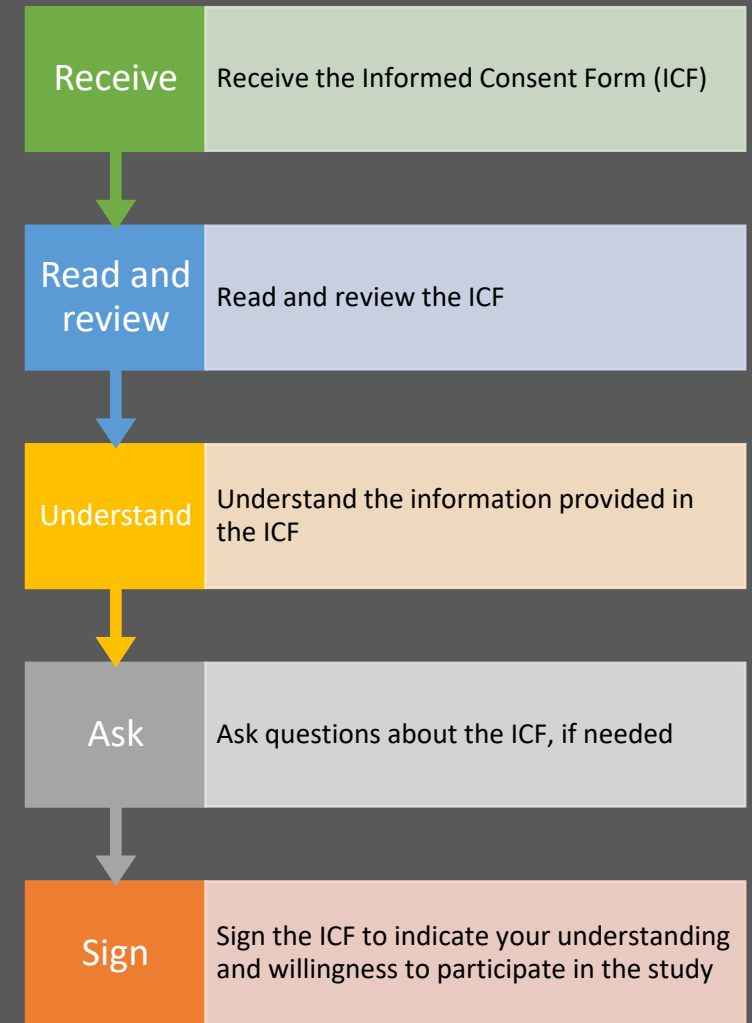
What is a placebo?

- An **inactive** substance or other intervention that looks the same as, and is given the same way as, an active drug or treatment being tested.
- The effects of the active drug or other intervention are compared to the effects of the placebo.

# What is an informed consent?



- Informed consent is a process that helps protect participants
- Before joining a clinical trial, you will be told what to expect as a participant, and all the things that might happen. For example, the principal investigator will explain possible side effects or other risks
- By signing an Informed Consent Form (ICF), you show that you have been told all the details of the study
- The ICF is NOT a contract
- You can leave the clinical trial at any time and for any reason
- Researchers must keep health and personal information private





# What questions should I ask my doctor?

- What is the purpose of the trial?
- What kind of treatments and tests are involved in the trial?
- Have similar studies already been done and what were the results?
- What are the possible risks or side effects of this treatment?
- How will my medical information and privacy be protected?
- How long will the trial last?
- How will the trial affect my daily life? How often will I need to come to the clinic?
- Who will fund this study?
- Do I have to pay for any of the treatments? What costs will my health insurance cover?
- Is there a chance I will receive placebo, and if so, will the new treatment be given should it prove to be better than current treatments?
- If I benefit from this therapy, will I be allowed to continue receiving it after the trial ends?
- Who will oversee my care while I am participating in the trial?
- Will I be able to take my regular medications while participating in the trial?



# Do I have to pay for any of the treatments?

- Most clinical trials are paid for by private industry or the federal government.
- Typically, you will NOT be asked to pay for participation in a clinical trial.
- You may receive compensation for clinical trial participation, e.g., for travel.
- Your health insurance may pay for 'standard of care' treatment costs.

Be suspect if you are asked to pay to participate in a clinical trial.

# Where do I find information about ongoing and upcoming clinical trials?

- Clinical Trials.gov
  - [www.clinicaltrials.gov](http://www.clinicaltrials.gov)
  - A service provided the National Institutes of Health (NIH)
  - Provides patients, family members and members of the public easy and free access to information on clinical trials
  - Includes federal and private studies nationwide
- Patient advocacy and support organizations such as NAAF
  - [www.naaf.org/research/clinical-trials](http://www.naaf.org/research/clinical-trials)
  - NAAF posts some clinical trial announcements for studies seeking to recruit people with alopecia areata
  - The NAAF website also links to Clinical Trials.gov

# ClinicalTrials.gov

- On landing page, you can search by:
  - Condition/disease
  - Other terms (such as study or sponsor names)
  - Country

NIH U.S. National Library of Medicine

**ClinicalTrials.gov**

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ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world.

Explore **442,235** research studies in all 50 states and in 221 countries.

See [listed clinical studies](#) related to the coronavirus disease (COVID-19)

ClinicalTrials.gov is a resource provided by the U.S. National Library of Medicine.

**IMPORTANT:** Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our [disclaimer](#) for details.

Before participating in a study, talk to your health care provider and learn about the [risks and potential benefits](#).

Find a study (all fields optional)

**Status** ⓘ

- Recruiting and not yet recruiting studies  
 All studies

**Condition or disease** ⓘ (For example: breast cancer)

Alopecia Areata X

**Other terms** ⓘ (For example: NCT number, drug name, investigator name)

X

**Country** ⓘ

X

**Search**

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List By Topic On Map Search Details

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Showing: 1-10 of 59 studies 10 studies per page Show/Hide Columns

Filters

Apply Clear

Status

Recruitment:

- Not yet recruiting
- Recruiting
- Enrolling by invitation
- Active, not recruiting
- Suspended
- Terminated
- Completed
- Withdrawn
- Unknown status†

Expanded Access:

Eligibility Criteria

Age:

Age Group:

- Child (birth–17)

Row	Saved	Status	Study Title	Conditions	Interventions	Locations
1	<input type="checkbox"/>	Recruiting	<a href="#">Study Evaluating Efficacy of Topical Squaric Acid Dibutyl Ester in Children and Adolescents With Alopecia Areata</a>	<ul style="list-style-type: none"> <li>Alopecia Areata</li> </ul>	<ul style="list-style-type: none"> <li>Drug: Placebo</li> <li>Drug: SADBE 5%</li> </ul>	<ul style="list-style-type: none"> <li>The University of Texas Health Science Center at Houston Houston, Texas, United States</li> </ul>
2	<input type="checkbox"/>	Recruiting	<a href="#">Study of DAXDILIMAB for the Treatment of Moderate-to-Severe Alopecia Areata</a>	<ul style="list-style-type: none"> <li>Alopecia Areata</li> </ul>	<ul style="list-style-type: none"> <li>Biological: Daxdilimab</li> </ul>	<ul style="list-style-type: none"> <li>Investigate MD, LLC Scottsdale, Arizona, United States</li> <li>Burke Pharmaceutical Research Hot Springs, Arkansas, United States</li> <li>First OC Dermatology Research, Inc. Fountain Valley, California, United States</li> <li>(and 17 more...)</li> </ul>
3	<input type="checkbox"/>	Recruiting	<a href="#">Needle-free Delivery of Intralesional Triamcinolone for Pediatric Alopecia Areata</a>	<ul style="list-style-type: none"> <li>Alopecia Areata</li> </ul>	<ul style="list-style-type: none"> <li>Combination Product: MedJet Device with intralesional triamcinolone</li> </ul>	<ul style="list-style-type: none"> <li>University Hospitals Cleveland Medical Center Cleveland, Ohio, United States</li> </ul>
4	<input type="checkbox"/>	Recruiting	<a href="#">Evaluate the Efficacy and Safety of SHR0302 in Adult Patients With Severe Alopecia Areata</a>	<ul style="list-style-type: none"> <li>Alopecia Areata</li> </ul>	<ul style="list-style-type: none"> <li>Drug: SHR0302</li> <li>Drug: Placebo</li> </ul>	<ul style="list-style-type: none"> <li>The Second Affiliated Hospital of Anhui Medical University Hefei, Anhui, China</li> <li>Beijing Tongren Hospital, Capital Medical University Beijing, Beijing, China</li> <li>China-Japan Friendship Hospital Beijing, Beijing, China</li> <li>(and 29 more...)</li> </ul>
5	<input type="checkbox"/>	Recruiting	<a href="#">A Study to Evaluate Efficacy and Safety of Deucravacitinib in Participants With Alopecia Areata</a>	<ul style="list-style-type: none"> <li>Alopecia Areata</li> </ul>	<ul style="list-style-type: none"> <li>Drug: Deucravacitinib</li> <li>Other: Placebo</li> </ul>	<ul style="list-style-type: none"> <li>University of California, Irvine (UCI) Health - UC Irvine Medical Center</li> </ul>

ClinicalTrials.gov

- Within returned results, use search boxes on right to refine results.
- Click on 'Study Title' to read details about the clinical trial.

# ClinicalTrials.gov

The study record page contains information about the study plan, participation criteria, locations, and more.

RECRUITING ⓘ

ClinicalTrials.gov Identifier: NCT05368103

## Study of DAXDILIMAB for the Treatment of Moderate-to-Severe Alopecia Areata

Information provided by Horizon Therapeutics Ireland DAC (Responsible Party)

Last Update Posted: 2023-02-09

Study

No Results Posted

Record History

Download

Print

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### Study Overview

Contacts and Locations

Participation Criteria

Study Plan

Collaborators and Investigators

Publications

More Information

### Study Overview

#### Brief Summary:

The purpose of this study is to assess the preliminary efficacy, safety, tolerability, PK, and PD of Daxdilimab in participants with moderate to severe AA, with  $\geq 50\%$  and  $\leq 95\%$  total scalp hair loss as defined by the SALT score at Screening and Day 1.

#### Detailed Description:

Approximately 30 participants will be enrolled to receive daxdilimab administered subcutaneously over 32 weeks. The maximum trial duration per participant is approximately 52 weeks, including up to 30 days for the screening period, 32 weeks for the open-label treatment period where participants will receive daxdilimab and approximately 16 weeks for the follow-up period. Safety evaluations will be performed regularly throughout t...

[Show more](#)

#### OFFICIAL TITLE

A Phase 2A, Open Label, Proof of Concept Trial of Daxdilimab for the Treatment of Moderate To Severe Alopecia Areata

#### CONDITIONS ⓘ

Alopecia Areata

#### STUDY TYPE ⓘ

Interventional

#### ENROLLMENT (ESTIMATED) ⓘ

30

#### INTERVENTION / TREATMENT ⓘ

Biological: Daxdilimab

#### PHASE ⓘ

Phase 2

#### OTHER STUDY ID NUMBERS ⓘ

HZNP-DAX-201

#### STUDY START (ACTUAL) ⓘ


2022-04-27

#### PRIMARY COMPLETION (ESTIMATED) ⓘ

2023-08-25

#### STUDY COMPLETION (ESTIMATED) ⓘ

2024-01-29

Resource links provided by the National Library of Medicine 

+

The FDA has a growing focus on diversity, inclusion & equity

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- FDA focus on health equity- [new draft guidance released April 2022](#)
  - Submit “race and ethnic diversity plan” early in clinical development
    - Set enrollment goals
    - Correct for underrepresentation in clinical trials
  - Establishes the ‘Diversity in Clinical Trials Initiative’ to support via public education and outreach

## **Diversity Plans to Improve Enrollment of Participants from Underrepresented Racial and Ethnic Populations in Clinical Trials**

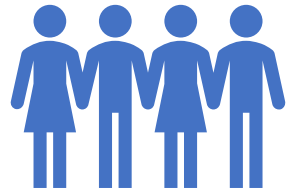
### **Guidance for Industry**

*DRAFT GUIDANCE*





# How does my participation in a clinical trial help the alopecia areata community?



## Helping Others

Participating in a clinical trial may help people with alopecia areata have better outcomes

Contribute to moving science forward



## Personal

Participating in a clinical trial may offer you access to the newest treatment

# Conclusions

- It is through clinical trials that safe and effective drugs, therapies and potentially a cure for alopecia areata will be discovered.
- By participating in a clinical trial, you play an important role in the fight to develop effective treatments and potentially identify a cure for alopecia areata.
- The decision to participate, however, is very personal and should be made only after speaking with your health care provider and other individuals you trust.
- You can stop participation in a clinical trial at any time if new concerns arise or you lose confidence that its potential benefits outweigh its risks.

# Thank you!

“Research participants are giving a gift to society.”

- Liz Martinez, Johns Hopkins Medicine Research Participant Advocate

