ClinicalTrials.gov
A service of the U.S. National Institutes of Health

Get to Know ClinicalTrials.gov

September 2015

Rebecca J. Williams, Pharm.D., MPH
Assistant Director, ClinicalTrials.gov
National Library of Medicine

Collaboration with the FDA Office of Minority Health

March 2010 Affordable Care Act- Section 10334 mandated creation of OMH across all HHS divisions

Mission

The Office of Minority Health aims to promote and protect the health of diverse populations through research and communication of regulatory science that addresses health disparities.

- *Goal 1- To improve and strengthen regulatory science informing the research and evaluation of sub-population data associations with race and ethnicity.*
- *Goal 2- To strengthen FDA capacity to address minority health and health disparities across the Agency*
- *Goal 3- To promote effective communication and the dissemination of information to the public, particularly underserved, vulnerable populations.*

FDA Office of Minority Health

Importance and Commitment to Diversity in Clinical Trials at FDA

"When a more diverse population participates in clinical trials, we increase the potential to know more about the extent to which different subgroups—males and females, young and old, people of various racial and ethnic backgrounds, and patients with differing comorbid diseases and conditions—might respond to a medical product. And when subgroup data are analyzed, we have available more information about the product that can be communicated to the public. The result is greater assurance in the safety and effectiveness of the medical products used by a diverse population."

Former FDA Commissioner Margaret Hamburg, August 2014

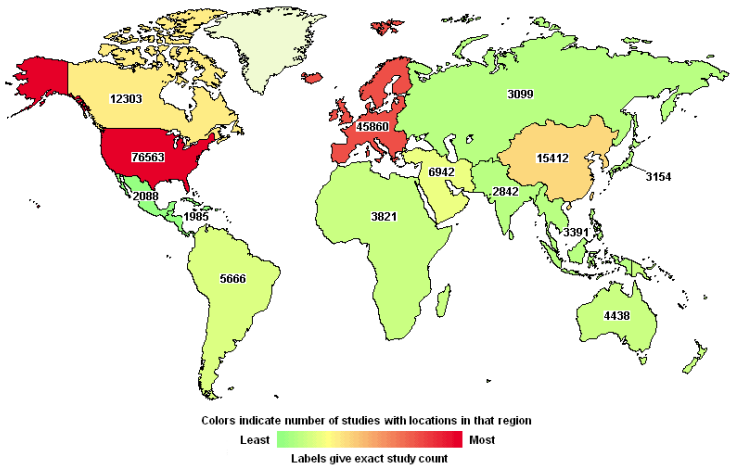
Outline for Presentation

- ClinicalTrials.gov Overview and Definitions
- ClinicalTrials.gov Content and Process
- Methods for Finding Trials
- ClinicalTrials.gov Results Database

About ClinicalTrials.gov

(as of September 2015)

- Clinical studies registry and results database
  - Over 199,000 studies (trials & observational studies)
  - Studies with locations in all 50 states and 190 countries
  - Privately and publicly funded studies involving humans
  - Study information submitted by sponsors
- Web Site & registry launched in February 2000
  - Results database, in September 2008
  - Over 18,000 studies with results
- Database updated nightly
- Usage
  - 179 million page views per month
  - 61,000 visitors per day



Why Conduct Clinical Trials?

- To obtain new and generalizable knowledge
- To provide evidence about treatments (benefits and risks) to inform medical decision making

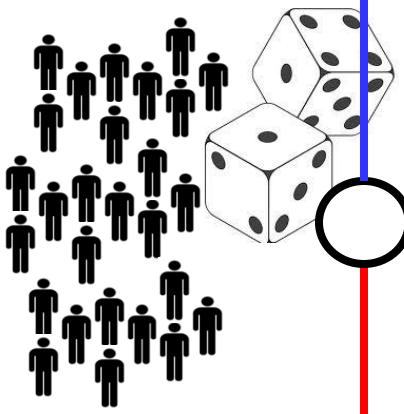
What is a Clinical Trial?

- A study that involves human participants to answer a specific research question about their health
- A protocol or research plan describes what will happen in the study (study visits, lab tests, etc.)
- Participants receive an intervention such as a drug (or placebo)
  - The protocol determines what a participant receives
  - When more than one intervention is being tested, the participant does not typically get to choose; assignment is random
- Participants are followed carefully for a period of time and evaluated for changes in health outcomes

Clinical Trial

Protocol

Intervention



A

Outcomes

B

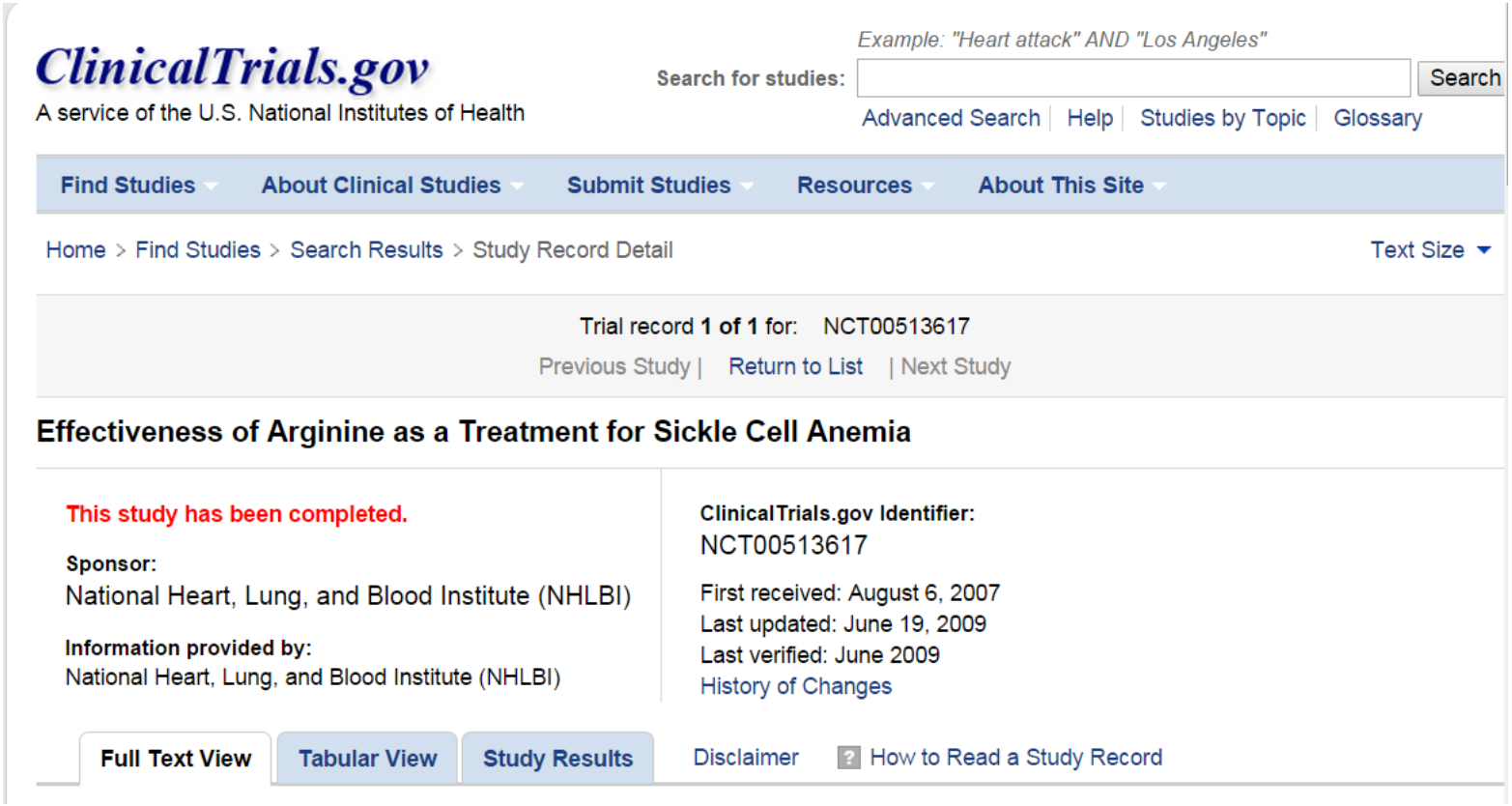
Outcomes

C

Outcomes

Example: Clinical Trial

- **Effectiveness of Arginine as a Treatment for Sickle Cell Anemia** (NCT00513617)



Clinical Trial Example
Adapted from NCT00513617

Arms	Assigned Interventions
Active Comparator **Low Dose 0.05 g/kg/day Arginine**	Drug: Arginine Depending on the weight of the child or adult, the patients took any where between 4-10 capsules 2 times a day. Patients weighing less than 45 kilograms were on the low dose active (or placebo) so the capsules were smaller. Patients greater than or equal to 45 kgs were on the high dose active or placebo, so these capsules were larger.
Active Comparator **High Dose 0.10 g/kg/day Arginine**	Drug: Arginine Depending on the weight of the child or adult, the patients took any where between 4-10 capsules 2 times a day. Patients weighing less than 45 kilograms were on the low dose active (or placebo) so the capsules were smaller. Patients greater than or equal to 45 kgs were on the high dose active or placebo, so these capsules were larger.
Placebo Comparator **Placebo (no Arginine)**	Drug: Placebo Depending on the weight of the child or adult, the patients took any where between 4-10 capsules 2 times a day. Patients weighing less than 45 kilograms were on the low dose active (or placebo) so the capsules were smaller. Patients greater than or equal to 45 kgs were on the high dose active or placebo, so these capsules were larger.

Clinical Trials Vary in Purpose

- Wide variety of populations
  - Healthy volunteers
  - People with the condition in various stages of disease and/or treatment (early → late)
- Wide range of goals
  - Assess level of toxicity, find appropriate dose, evaluate drug interactions
  - Study the disease process
  - Evaluate if an intervention can prevent or treat disease
  - Compare known treatments to each other

Other Types of Research

- Observational studies
  - A study that uses a protocol or research plan to evaluate a specific research question in human participants
  - Participants receive "usual medical care" (their treatment is not determined by the protocol)
  - Participants are "observed" over time and health outcomes assessed

Observational Study

Routine Medical Care

Protocol

A

Outcomes

B

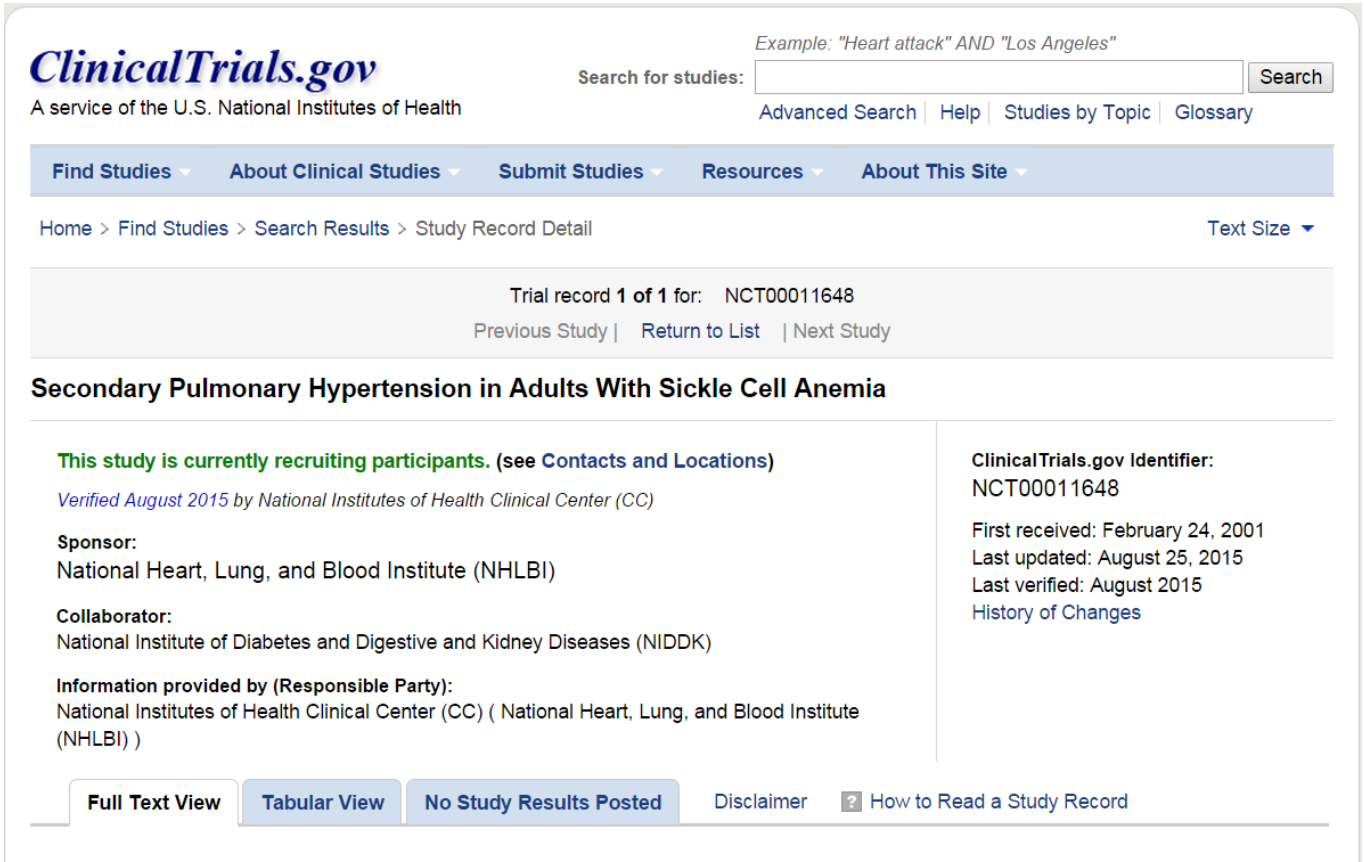
Outcomes

C

Outcomes

Example: Observational Study

- Secondary Pulmonary Hypertension in Adults With Sickle Cell Anemia (NCT00011648)



Observational Study Example
Adapted from NCT00011648

- Men and women 18 years of age and older with sickle cell anemia
  - Initial assessment (medical history, physical exam, blood collection, echocardiogram)
- A study nurse will contact participants two times a month for 2 months, then once every 3 months for 3 years
  - Telephone interview with questions about health and health-related events (hospitalizations or emergency room visits)

ClinicalTrials.gov Content and Process

Why Register a Study and Submit Results on ClinicalTrials.gov?

- Ethical and Scientific Rationale
  - Responsibility to research participants, patients, and the public
  - Research integrity
  - Evidence-based medicine
- Required by various policies and laws

Key U.S. Policies and Laws

- **Journal editors** require registration of clinical trials (2005)

- **U.S. Federal Laws**
  - FDAMA 113 (1997): registration of clinical trials of investigational drugs for serious and life-threatening conditions
  - FDAAA 801 (2007): registration & results for clinical trials of drugs and devices (early/Phase 1 trials excluded)
    - Results reporting only required for trials of "approved" products (but proposed to expand to include unapproved)

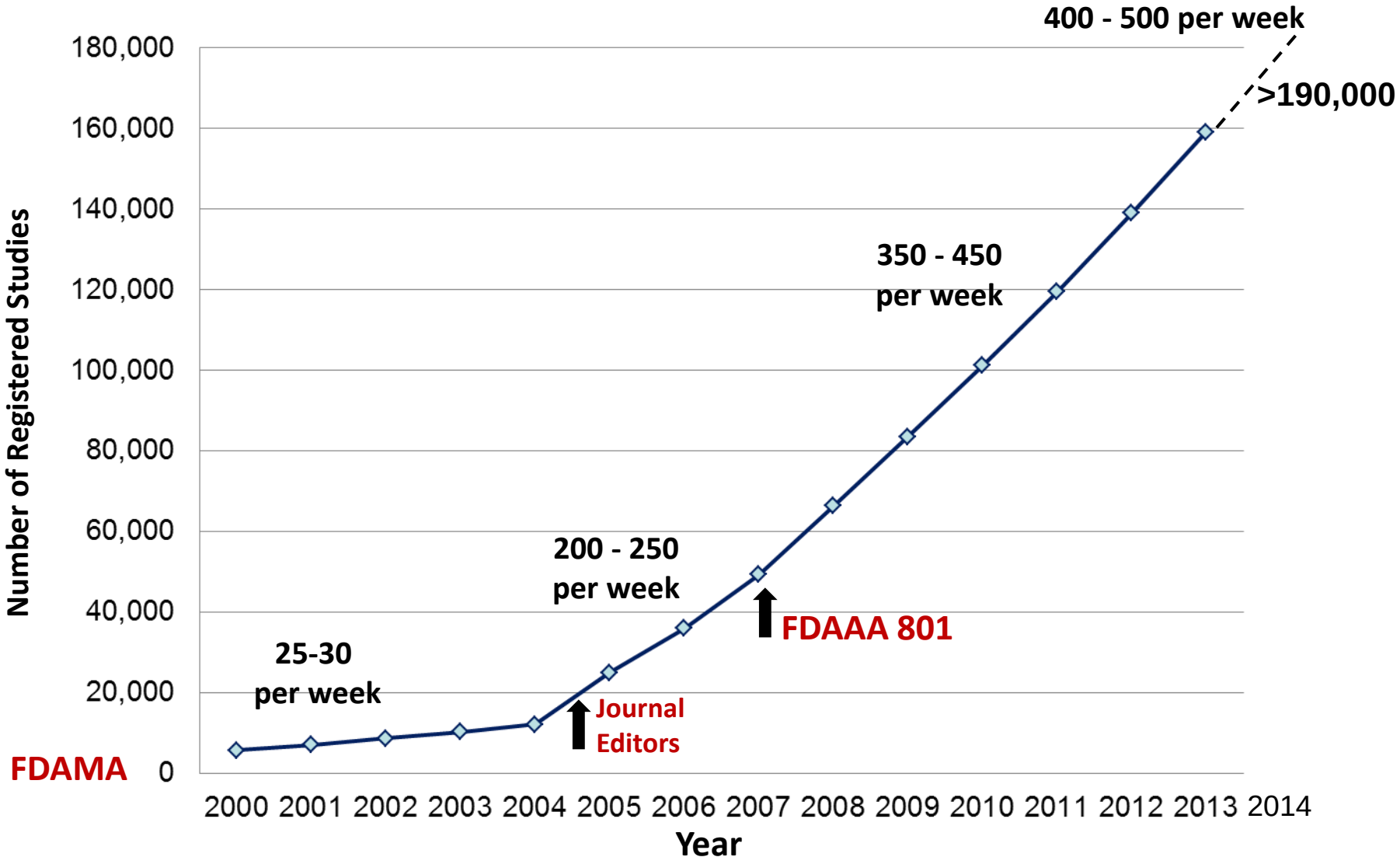
- **NIH** encourages registration & results for all NIH-funded clinical trials
  - Proposal issued by NIH in Nov 2014 to make this a requirement

FDAMA 113 = Section 113 of the Food and Drug Administration Modernization Act of 1997; **FDAAA 801** = Section 801 of the Food and Drug Administration Amendments Act of 2007

Complete information at ClinicalTrials.gov History, Policies, and Laws: https://clinicaltrials.gov/ct2/about-site/history

Studies Registered Over Time

(as of September 2015)



ClinicalTrials.gov Content

(as of September 24, 2015)

	Registration	Results
Total	**199,165†**	**18,463**
Type of Trial		
Observational	37,760 (18%)	1,163 (6%)
Interventional*	160,497 (80%)	17,300 (93%)
- Drug or Biologic	101,366	14,116
- Behavioral, Other	44,302	2,680
- Surgical Procedure	17,371	890
- Device	17,044	1,868

† Includes 321 Expanded Access study records and 587 applicable device clinical trials that submitted a "delayed posting" under FDAAA

* Intervention types not additive; study record may include more than one type of intervention

ClinicalTrials.gov Content

(as of September 24, 2015)

	Registration	Results
Total	**199,165**	**18,463**
Funder Type†		
Universities, foundations	97,426	3,399
Industry	74,748	12,523
U.S. NIH	24,482	2,241
U.S. Federal Gov.t	4,640	589
Location		
Non-U.S. Only	91,039 (46%)	--
U.S. Only	76,066 (38%)	--
Not specified	20,360 (10%)	--
Both U.S. and Non-U.S.	11,700 (6%)	--

† Funder types not additive; study record may include more than one type of funder

Content of a Study Record

(One Record per Unique Study Protocol – *Single NCT Number*)

- Protocol section
  - Submitted at trial start; updated throughout trial lifecycle
  - Summarizes information from trial protocol: e.g.,
    - Condition
    - Interventions
    - Study Design
  - Includes recruitment information (e.g., eligibility, locations)
- Results section
  - Submitted after trial completion/termination
  - Summarizes trial results
    - Participant flow
    - Baseline characteristics
    - Outcome measures (including statistical analyses)
    - Adverse events

Example: Observational Study

- Secondary Pulmonary Hypertension in Adults With Sickle Cell Anemia (NCT00011648)

Highlights

Study Title

Recruitment Status

Sponsor & Funders

NCT Number

Dates

Highlights

Study Design

Who can Participate

Who to Contact

Secondary Pulmonary Hypertension in Adults With Sickle Cell Anemia

Study Type: Observational
ClinicalTrials.gov Identifier: NCT00011648
Estimated Enrollment: 1200
Study Start Date: February 2001
Outcome Measures: Prevalence of secondary pulmonary hypertension …

Eligibility

Ages Eligible for Study:	18 Years and older
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	Yes

Criteria

- INCLUSION CRITERIA:

All volunteer subjects must be at least 18 years of age and must be able to provide informed, written consent for participation in this study. Decisional impaired subjects will not be included in this study because it does not offer the prospect of direct benefit.

Sickle Cell Patients:

Male and females over 18 years of age.

Diagnosis of sickle cell disease (electrophoretic documentation of SS, SC, or S-beta thallassemia genotype is required).

Contacts and Locations

Choosing to participate in a study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff using the Contacts provided below. For general information, see Learn About Clinical Studies.

Please refer to this study by its ClinicalTrials.gov identifier: NCT00011648

Locations

United States, Maryland

National Institutes of Health Clinical Center, 9000 Rockville Pike — **Recruiting**
Bethesda, Maryland, United States, 20892
Contact: For more information at the NIH Clinical Center contact Patient Recruitment and Public Liaison Office (PRPL) 800-411-1222 ext TTY8664111010 prpl@mail.cc.nih.gov

ClinicalTrials.gov Process Overview

- Information submitted to ClinicalTrials.gov by Responsible Party of study (Sponsor or Principal Investigator)
- A record represents a single trial, even if more than 1 location
- Basic review criteria must be met before public posting
  - Automated rules then manual review by ClinicalTrials.gov staff
- Each record is:
  - Assigned a unique identifier (NCT Number)
  - Expected to be corrected/updated throughout life cycle
  - All changes tracked in public Archive site
- We add links to other useful resources about the condition evaluated or product studied (examples: Medline Plus; Genetics Home Reference; PubMed)

Review by ClinicalTrials.gov

- Evaluate whether study information is complete and generally understandable
  - Internal consistency and logic; complete and meaningful entries; formatting
- Posting of a study on ClinicalTrials.gov does not mean that it is endorsed by the National Institutes of Health (NIH)
- Choosing to participate in a study is an important personal decision. Talk with your doctor and family/friends about deciding to join a study.

Who “Approves” a Clinical Trial?

- Clinical trials of drugs in the U.S. are reviewed, approved, and monitored by an ethics committee (known as an institutional review board or IRB)
- Purpose is to ensure that study meets ethical standards and that rights and welfare of participants are protected
  - Informed consent
- Clinical trials recruiting on ClinicalTrials.gov must indicate that they have IRB approval

Sample Uses of ClinicalTrials.gov

- Identify trials of potential interest for an individual or user community
- Track progress of a specific trial and availability of summary results
- Identify completed or ongoing trials for specific conditions/interventions
  - Supplements a literature review
- Identify researchers and/or centers of relevance to specific conditions/interventions

ClinicalTrials.gov Visitors by Role (2014)

Role	% Respondents N=5,397
Patient	27%
Scientist/Researcher	19%
Family or Friend of Patient	13%
Health Care Provider (e.g., nurse, physician)	8%
Other	8%
Clinical Trial Staff	7%
Clinical Research Support (e.g. regulatory affairs)	5%
Healthy Person	4%
Student/Educator	4%
Medical Communications	3%
Librarian or Information Professional	2%
Institutional Review Board (IRB) or Ethics Committee Member	1%

Source: ForeSee American Customer Satisfaction Index Survey Results: 1 Jan to 31 Dec 2014

Methods for Finding Trials

Methods for Finding Trials

- Basic Search
- Advanced Search - allows for a focused search
  - https://clinicaltrials.gov/ct2/search/advanced
- See Studies by Topic
  - Conditions/Rare Diseases, Drugs, Dietary Supplements, Sponsor/Collaborators, Locations
    - https://clinicaltrials.gov/ct2/search/browse
- See Studies on Map
  - https://clinicaltrials.gov/ct2/search/map

Demonstration

ClinicalTrials.gov
A service of the U.S. National Institutes of Health

ClinicalTrials.gov is a registry and results database of publicly and privately supported clinical studies of human participants conducted around the world. Learn more about clinical studies and about this site, including relevant history, policies, and laws.

Find Studies | About Clinical Studies | Submit Studies | Resources | About This Site

ClinicalTrials.gov currently lists 194,735 studies with locations in all 50 States and in 190 countries.

Text Size

Search for Studies

Example: "Heart attack" AND "Los Angeles"

Search

Advanced Search | See Studies by Topic
See Studies on a Map

Search Help

- How to search
- How to find results of studies
- How to read a study record

Locations of Recruiting Studies

- Non-U.S. Only (53%)
- U.S. Only (41%)
- Both U.S. and Non-U.S. (6%)

Total N = 36,062 studies
(Data as of July 16, 2015)

- See more trends, charts, and maps

For Patients and Families

- How to find studies
- See studies by topic
- Learn about clinical studies
- Learn more…

For Researchers

- How to submit studies
- Download content for analysis
- About the results database
- Learn more…

For Study Record Managers

- Why register?
- How to register your study
- FDAAA 801 requirements
- Learn more…

Learn More

- Tutorials for using ClinicalTrials.gov
- Glossary of common site terms
- For the Press
- Using our RSS Feeds

HOME | RSS FEEDS | SITE MAP | TERMS AND CONDITIONS | DISCLAIMER | CONTACT NLM HELP DESK

Copyright | Privacy | Accessibility | Viewers & Players | Freedom of Information Act | USA.gov
U.S. National Library of Medicine | U.S. National Institutes of Health | U.S. Department of Health and Human Services

See Studies by Topic

Select a topic:

Conditions

Alphabetical (A–Z)

By Category

Rare Diseases

Alphabetical (A–Z)

Drug Interventions

Alphabetical (A–Z)

By Category

Dietary Supplements

Alphabetical (A–Z)

By Category

See Conditions by First Letter

A B C D E F G H I J K L M N O P Q R S T U V W X Y Z Other All

ACTH Syndrome, Ectopic 8 studies

ACTH-Secreting Pituitary Adenoma 4 studies

ADULT Syndrome 1 study

AIDS Dementia Complex 30 studies

AIDS-Associated Nephropathy 6 studies

AIDS-Related Complex 280 studies

AIDS-Related Opportunistic Infections 177 studies

AL Amyloidosis 131 studies

AML With Myelodysplasia-related Features 29 studies

Abdomen, Acute 15 studies

Abdominal Abscess 28 studies

Abdominal Aortic Aneurysm 211 studies

Abdominal Injuries 20 studies

See Studies by Topic

Select a topic:

Conditions

Alphabetical (A–Z)

By Category

Rare Diseases

Alphabetical (A–Z)

Drug Interventions

Alphabetical (A–Z)

By Category

Dietary Supplements

Alphabetical (A–Z)

By Category

Sponsor/Collaborators

See Conditions by First Letter

A B C D E F G H I J K L M N O P Q R S T U V W X Y Z Other All

SAPHO Syndrome 2 studies

Sacrococcygeal Teratoma 1 study

Sacroiliitis 13 studies

Salivary Gland Diseases 271 studies

Salivary Gland Neoplasms 73 studies

Salmonella Infections 29 studies

Sandhoff Disease 15 studies

Sarcoglycanopathies 2 studies

Sarcoidosis 142 studies

Sarcoidosis, Pulmonary 22 studies

Sarcoma 1,222 studies

Sarcoma Botryoides 1 study

Sarcoma, Alveolar Soft Part 37 studies

Sarcoma, Clear Cell 30 studies

Shoulder Pain 147 studies

Shprintzen-Goldberg Craniosynostosis Syndrome 1 study

Shwachman-Diamond Syndrome 12 studies

Shy-Drager Syndrome 56 studies

Sialadenitis 3 studies

Sialorrhea 25 studies

Sick Sinus Syndrome 43 studies

Sickle Beta Thalassemia 12 studies

Sickle Cell Anemia 417 studies

Sickle Cell Trait 10 studies

Sideroblastic Anemia Pyridoxine-refractory Autosomal Recessive 72 studies

Siderosis 13 studies

Sigmoid Neoplasms 1 study

Signs and Symptoms 17.9K studies

Signs and Symptoms, Digestive 1,673 studies

Signs and Symptoms, Respiratory 1,014 studies

Silicosis 7 studies

Silver-Russell Syndrome 2 studies

Single Ventricular Heart 46 studies

Sinonasal Undifferentiated Carcinoma 1 study

Sinus Cancer 4 studies

Sinus Thrombosis, Intracranial 2 studies

417 studies found for: "Sickle Cell Anemia"

Modify this search | How to Use Search Results

List | By Topic | On a Map | Search Details

+ Show Display Options

Download | Subscribe to RSS

Include only open studies | Exclude studies with Unknown status

Rank	Status	Study
1	Recruiting	Microvascular Blood Flow in Sickle Cell Anemia **Conditions:** Sickle Cell Disease; Sickle Cell Anemia **Interventions:** Drug: regadenoson infusion with contrast-enhanced ultrasound; Procedure: contrast-enhanced ultrasound
2	Completed	A Study of the Efficacy and Safety of ICA-17043 (With or Without Hydroxyurea) in Patients With Sickle Cell Anemia. **Conditions:** Sickle Cell Disease; Sickle Cell Anemia **Interventions:** Drug: Low Dose ICA-17043; Drug: High dose ICA-17043; Drug: Placebo
3	Recruiting	Secondary Pulmonary Hypertension in Adults With Sickle Cell Anemia **Conditions:** Pulmonary Hypertension; Sickle Cell Anemia; Sickle Cell Disease **Intervention:**
4	Completed	L-Glutamine Therapy for Sickle Cell Anemia **Conditions:** Sickle Cell Anemia; Thalassemia

417 studies found for: "Sickle Cell Anemia"

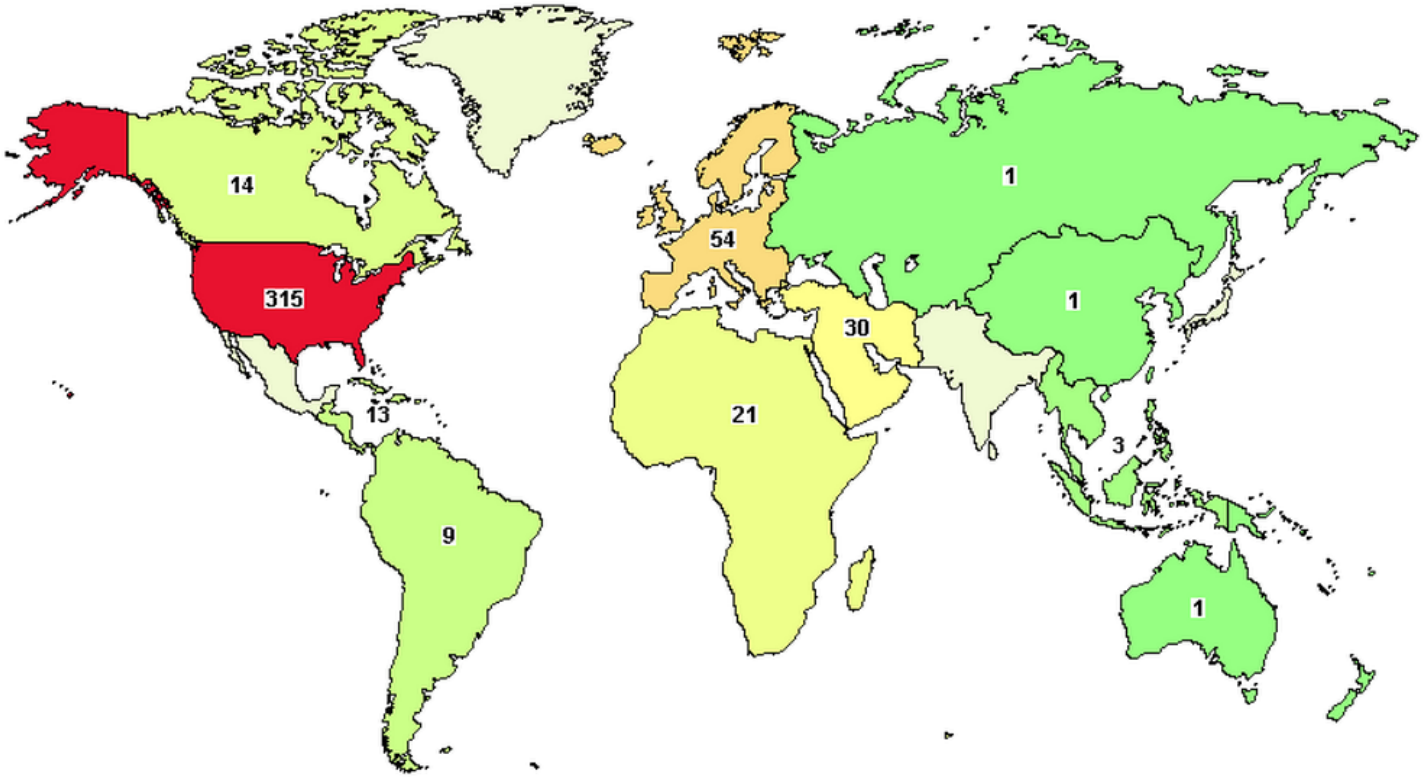
Modify this search | How to Use Search Results

List | By Topic | On a Map | Search Details

417 studies found, shown on map.

A similar map is available for all studies in ClinicalTrials.gov

Click on the map below to show a more detailed map (when available) or search for studies (when map not available).



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Fill in any or all of the fields below. Click on the label to the left of each search field for more information or read the Help

Expert Search

Search Terms: Search Help

Recruitment: -- Recruiting ☑ Exclude Unknown status

Study Results: All Studies

Study Type: Interventional Studies

Targeted Search

Conditions: "Sickle Cell Anemia"

Interventions:

Title Acronym/Titles:

Outcome Measures:

Sponsor/Collaborators: ☐ Exact match

Sponsor (Lead): ☐ Exact match

Study IDs:

Locations

State 1: --- Optional ---

Country 1: United States

State 2: --- Optional ---

Country 2: --- Optional ---

18 studies found for: Recruiting | Exclude Unknown | Interventional Studies | "Sickle Cell Anemia" | United States | NIH

Modify this search | How to Use Search Results

List | By Topic | On a Map | Search Details

+ Show Display Options

Download | Subscribe to RSS

Exclude studies with Unknown status

Rank	Status	Study
1	Recruiting	A Phase II Trial of Regadenoson in Sickle Cell Anemia **Condition:** Sickle Cell Anemia **Interventions:** Drug: Regadenoson; Drug: Placebo
2	Recruiting	Bone Marrow Transplantation in Young Adults With Severe Sickle Cell Disease **Condition:** Sickle Cell Disease **Intervention:** Other: Biological: Bone Marrow Transplant
3	Recruiting	Hydroxyurea to Prevent Brain Injury in Sickle Cell Disease **Conditions:** Sickle Cell Disease; Stroke **Interventions:** Drug: Hydroxyurea; Drug: Placebo
4	Recruiting	Nonmyeloablative Peripheral Blood Mobilized Hematopoietic Precursor Cell Transplantation for Sickle Cell Disease and Beta-thalassemia in People With Higher Risk of Transplant Failure **Conditions:** Sickle Cell Disease; Thalassemia; Stem Cell Transplantation; Graft vs Host Disease

18 studies found for: Recruiting | Exclude Unknown | Interventional Studies | "Sickle Cell Anemia" | United States | NIH

Modify this search | How to Use Search Results

List | By Topic | On a Map | Search Details

– Hide Display Options

Download Subscribe to RSS

Display Options

Study Details	Participant Details	Identifiers	Dates
☑ Condition	☐ Number Enrolled	☐ NCT Number	☐ Study Start
☑ Intervention	☐ Gender	☐ Other IDs	☐ Primary Completion
☐ Study Type	☐ Age Group	☐ Title Acronym	☐ Study Completion
☐ Phase			☐ First Received
☐ Sponsor/Collaborators			☐ Last Updated
☐ Funder Type			☐ Last Verified
☐ Study Design			☐ Results First Received
☐ Outcome Measures			

Update Display Cancel

☑ Exclude studies with Unknown status

Rank	Status	Study
1	Recruiting	A Phase II Trial of Regadenoson in Sickle Cell Anemia **Condition:** Sickle Cell Anemia **Interventions:** Drug: Regadenoson; Drug: Placebo

Search Results Tools

- RSS Feed
  - Receive automatic updates on a specific search
  - https://clinicaltrials.gov/ct2/resources/rss
- Download Search Results
  - Choose study fields to include
  - Select file format (tab- and comma-separated values format is appropriate for a spreadsheet)
  - https://clinicaltrials.gov/ct2/resources/download

417 studies found for: "Sickle Cell Anemia"
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Other Considerations for Finding Trials

- There are many organizations that exist to provide support to people with a specific disease or condition and their families/friends
  - May have resources that are more targeted for your needs
  - Often includes ClinicalTrials.gov information
- Other organizations aim to “match” people to trials (and may require personal information)
  - BreastCancerTrials.org
  - ResearchMatch.org
  - Others …

Possible Questions to Ask if Considering Participating in Research

- What is being studied?
- Why do researchers think the intervention may or may not be effective? Has it been tested before?
- What are the possible interventions that I might receive in trial? How will this be determined?
- How do the possible risks, side effects, benefits compare to my current treatment?

https://clinicaltrials.gov/ct2/about-studies/learn

Possible Questions to Ask if Considering Participating in Research

- What tests and procedures are involved?
- How often will I have to visit hospital/clinic?
  - Will an overnight stay be required?
- How long will study last?
- Who is funding the study? Who will pay costs?
- Will I received the study results?
- Who will oversee my medical care?
- What are my options if I am injured in the study?

https://clinicaltrials.gov/ct2/about-studies/learn

ClinicalTrials.gov Results Database

ClinicalTrials.gov Results Database

- Current requirements for results posting:
  - U.S. Law (FDAAA 801)
    - Clinical trials of approved drugs, biologics, devices
      - Excluded: Phase 1 drug & small device feasibility trials
    - Completed after December 2007
- Additional requirements may be coming*:
  - DRAFT NIH Policy: All NIH-funded clinical trials
  - Rulemaking for FDAAA: Include unapproved products
- Approximately half of studies with results on ClinicalTrials.gov are not yet published

*http://www.nih.gov/news/health/nov2014/od-19.htm

ClinicalTrials.gov Results Database: Basic information – reported by arm

- Participant Flow
  - Number Started
  - Number Completed
- Baseline Characteristics
  - Number Analyzed
  - Age and gender
  - Other relevant measures (e.g., disease history)
- Primary and Secondary Outcome Measures
  - Outcome Data
  - Statistical analyses (if any)
- Serious and Other Adverse Events
  - Total Number of Events
  - Specific Events by Organ Class

Studies with Results in ClinicalTrials.gov

43	Completed	Gum Arabic as Fetal Hemoglobin Agent in Sickle Cell Anemia **Condition:** Sickle Cell Anemia **Intervention:** Dietary Supplement: Gum Arabic
44	Completed Has Results	Prasugrel Versus Placebo in Adult Sickle Cell Disease **Condition:** Sickle Cell Anemia **Interventions:** Drug: Prasugrel; Drug: Placebo
45	Recruiting	Retroviral Vector Mediated Globin Gene Transfer to Correct Sickle Cell Anemia or Thalassemia **Conditions:** Sickle Cell Anemia; Thalassemia **Intervention:** Genetic: Gene Therapy
46	Completed	Tricuspid Regurgitant Jet Velocity as an Independent Marker for Mortality in Sickle Cell Anemia **Conditions:** Sickle Cell Anemia; Pulmonary Hypertension **Intervention:** Other: Data Collection

Example of Study with Results

Prasugrel Versus Placebo in Adult Sickle Cell Disease

This study has been completed.

Sponsor:
Eli Lilly and Company

Collaborator:
Daiichi Sankyo Inc.

Information provided by (Responsible Party):
Eli Lilly and Company

ClinicalTrials.gov Identifier:
NCT01167023

First received: July 20, 2010
Last updated: April 9, 2012
Last verified: April 2012
History of Changes

Full Text View | Tabular View | **Study Results** | Disclaimer | How to Read a Study Record

Results First Received: April 9, 2012

Study Type:	Interventional
Study Design:	Allocation: Randomized; Endpoint Classification: Safety Study; Intervention Model: Parallel Assignment; Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor); Primary Purpose: Treatment
Condition:	**Sickle Cell Anemia**
Interventions:	Drug: Prasugrel Drug: Placebo

Participant Flow

Adapted from NCT01167023

Overall Study

	Placebo	5 mg Prasugrel	7.5mg Prasugrel
Started	**21**	**41**	**0**
Received at least 1 dose of study drug	**19**	**41**	**0**
Completed	**18**	**39**	**0**
Not Completed	**3**	**2**	**0**
Lost to Follow-up	1	1	0
Screen Failure	1	0	0
Sponsor Decision	1	0	0
Withdrawal by Subject	0	1	0

See full study record for complete information: https://clinicaltrials.gov/ct2/show/NCT01167023

Baseline Measures

Adapted from NCT01167023

Baseline Measures

	Placebo	5 mg Prasugrel	Total
Number of Participants	21	41	62
Age **[units: years]** **Mean (Standard Deviation)**	31.52 (8.20)	32.88 (8.60)	32.42 (8.42)
Gender **[units: participants]**			
Female	9	21	30
Male	12	20	32
Ethnicity (NIH/OMB) **[units: participants]**			
Hispanic or Latino	0	1	1
Not Hispanic or Latino	21	40	61
Unknown or Not Reported	0	0	0
Race (NIH/OMB) **[units: participants]**			
American Indian or Alaska Native	0	0	0
Asian	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	21	40	61
White	0	1	1
More than one race	0	0	0
Unknown or Not Reported	0	0	0

See full study record for complete information: https://clinicaltrials.gov/ct2/show/NCT01167023

Outcome Measures

Adapted from NCT01167023

Primary Outcome

Measure Title	Percentage of Participants With Hemorrhagic Events Requiring Medical Intervention During the Treatment Duration
Measure Description	A hemorrhagic event requiring medical intervention. Medical intervention was defined as any medical attention resulting in therapy or further investigation during the 30-day treatment duration.
Time Frame	Baseline through 30 days

Measured Values

	Placebo	5 mg Prasugrel
Number of Participants Analyzed	19	41
[units: percentage of participants]	0	0

See full study record for complete information: https://clinicaltrials.gov/ct2/show/NCT01167023

Serious Adverse Events

Adapted from NCT01167023

Serious Adverse Events

	Placebo	5 mg Prasugrel
Total Serious Adverse Events # participants affected/at risk	**4/19 (21.05%)**	**8/41 (19.51%)**
Blood and lymphatic system disorders		
Pancytopenia	1/19 (5.26%)	0/41 (0.00%)
Congenital, familial and genetic disorders		
Sickle cell anemia	0/19 (0.00%)	1/41 (2.44%)
Sickle cell anemia with crisis	1/19 (5.26%)	2/41 (4.88%)
Ear and labyrinth disorders		
Vertigo	0/19 (0.00%)	1/41 (2.44%)
General Disorders		
Pain ….	1/19 (5.26%)	0/41 (0.00%)

See full study record for complete information: https://clinicaltrials.gov/ct2/show/NCT01167023

Linking P

Safety and Efficacy Study … Aeruginosa (AIR-CF2)

This study has been completed.

Sponsor:
Gilead Sciences

Information provided by:
Gilead Sciences

Full Text View | Tabular Vie

Purpose

The purpose of this study was to eval… lung infection due to Pseudomonas a…

Condition

Cystic Fibrosis

More Information

No publications provided by Gilead Sc…

Additional publications automatically i…

McCoy KS, Quittner AL, Oermann CM… aeruginosa in cystic fibrosis. Am J Respir Crit Care Med. 2008 Nov 1;178(9):921-8. Epub 2008 Jul 24.

NC… Publ… US Nation… National I…

Display…

Am J Res…

Inhale… fibros…

McCoy K…

Auth…

Abstra…

RATIO… (CF) on… random…

OBJEC… with CF…

METHO… =6 yr; … with 75… endpoi… include… functio… PA wer…

MEASU… antipseudomonal antibiotics for symptoms of pulmonary exacerbation by 21 days, compared with placebo (AZLI, 92 d; placebo, 71 d; P = 0.007). AZLI improved mean CFQ-R respiratory scores (5.01 points, P = 0.02), FEV(1) (6.3%, P = 0.001), and sputum PA density (-0.66 log(10) cfu/g, P = 0.006) compared with placebo; no AZLI dose-response was observed. Adverse events reported for AZLI and placebo were comparable and consistent with CF lung disease. Susceptibility of PA to aztreonam at baseline and end of therapy were similar.

CONCLUSIONS: AZLI was effective in patients with CF using frequent TIS therapy. AZLI delayed time to need for inhaled or intravenous antipseudomonal antibiotics, improved respiratory symptoms and pulmonary function, and was well tolerated. Clinical trial registered with www.clinicaltrials.gov (NCT 00104520).

Inhaled Aztreonam Lysine for Chronic Airway *Pseudomonas aeruginosa* in Cystic Fibrosis

Karen S. McCoy[1], Alexandra L. Quittner[2], Christopher M. Oermann[3], Ronald L. Gibson[4], George Z. Retsch-Bogart[5], and A. Bruce Montgomery[6]

[1]Ohio State University, Columbus, Ohio; [2]University of Miami, Coral Gables, Florida; [3]Baylor College of Medicine, Houston, Texas; [4]Children's Hospital and Regional Medical Center, Seattle, Washington; [5]University of North Carolina at Chapel Hill, Chapel Hill, North Carolina; and [6]Gilead Sciences, Inc., Seattle, Washington

Rationale: The effectiveness and safety of aztreonam lysine for inhalation (AZLI) in patients with cystic fibrosis (CF) on maintenance treatment for *Pseudomonas aeruginosa* (PA) airway infection was evaluated in this randomized, double-blind, placebo-controlled study.
Objectives: To evaluate the safety and efficacy of inhaled aztreonam lysine in controlling PA infection in patients with CF.
Methods: After randomization and a 28-day course of tobramycin inhalation solution (TIS), patients (n = 211; ≥6 yr; ≥3 TIS courses within previous year; FEV_1 ≥ 25% and ≤75% predicted values) were treated with 75 mg AZLI or placebo, twice or three times daily for 28 days, then monitored for 56 days. The primary efficacy endpoint was time to need for additional inhaled or intravenous antipseudomonal antibiotics. Secondary endpoints included changes in respiratory symptoms (CF Questionnaire-Revised [CFQ-R] Respiratory Scale), pulmonary function (FEV_1), and sputum PA density. Adverse events and minimum inhibitory concentrations of aztreonam for PA were monitored.
Measurements and Main Results: AZLI treatment increased median time to need for additional antipseudomonal antibiotics for symptoms of pulmonary exacerbation by 21 days, compared with placebo (AZLI, 92 d; placebo, 71 d; $P = 0.007$). AZLI improved mean CFQ-R respiratory scores (5.01 points, $P = 0.02$), FEV_1 (6.3%, $P = 0.001$), and sputum PA density (-0.66 $\log_{10}$ cfu/g, $P = 0.006$) compared with placebo; no AZLI dose–response was observed. Adverse events reported for AZLI and placebo were comparable and consistent with CF lung disease. Susceptibility of PA to aztreonam at baseline and end of therapy were similar.
Conclusions: AZLI was effective in patients with CF using frequent TIS therapy. AZLI delayed time to need for inhaled or intravenous antipseudomonal antibiotics, improved respiratory symptoms and pulmonary function, and was well tolerated.
Clinical trial registered with www.clinicaltrials.gov (NCT 00104520).

AT A GLANCE COMMENTARY

Scientific Knowledge on the Subject

Cystic fibrosis is a chronic disease often involving endobronchial infection with *Pseudomonas aeruginosa*, which is difficult to treat.

What This Study Adds to the Field

Safety and efficacy data on inhaled aztreonam show that this new formulation may be an alternative treatment option for patients with cystic fibrosis and chronic *P. aeruginosa* infection.

older patients, the most common pathogen in CF airway infections is *Pseudomonas aeruginosa* (PA); these infections are associated with an accelerated decline in pulmonary function and increased mortality (2–4).

Over the past 15 years, management of patients with CF has improved (1, 2, 5–8). However, antimicrobial treatment options for chronic PA airway infections remain limited and additional therapies are needed to augment improvements in clinical outcomes.

Aztreonam lysine for inhalation (AZLI) is an aerosolized formulation of the monobactam antibiotic aztreonam and lysine (9). The intravenous aztreonam formulation contains arginine, which can cause airway inflammation after chronic inhalation therapy in patients with CF (10, 11). The study described herein included patients with CF who frequently used antibiotics for PA

Clarifications about Results Requirements

- Summary results at the end of the trial
- Information currently targeted at readers of the medical literature
  - "Tables" of information; "just the facts"
  - No conclusions or discussion
- Results submission is not required for registered studies that are not subject to law (FDAAA 801)

Additional Resources

- ClinicalTrials.gov Online Training – brief animated tutorials
  - http://www.nlm.nih.gov/bsd/viewlet/ct/
  - Topics currently available: Basic Search, Advanced Search, Customize Your Display, Downloading Search Results, Modify a Search, RSS Feed Setup for a Search, Study Record Details, Using the ClinicalTrials.gov Results Database

- Find Studies: https://www.clinicaltrials.gov/ct2/search/index

- Learn About Clinical Studies
  - https://clinicaltrials.gov/ct2/about-studies/learn

Thank you!

Questions?

Contact us at: register@clinicaltrials.gov