

CheckRare

Rare and Genetic Disease Network

CME / CE

Rare Disease Clinical Trials

Focus on Rare Hematologic Clinical Trials

Morie A Gertz MD: Mayo Clinic; Rochester, MN

Rare Hematologic Conditions

Definition

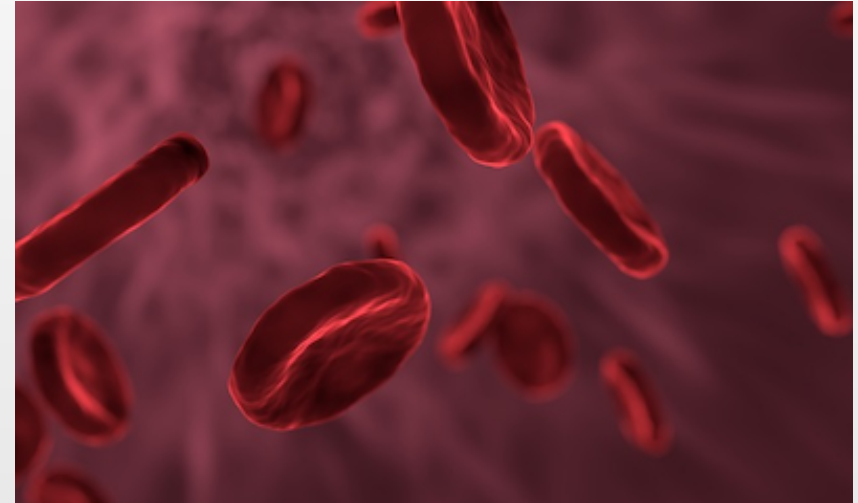
- A rare disease is any disease affected less than 200,000 people in the United States

Why is a Rare Disease Designation Important?

- Orphan Drug Act
- No one left behind

Common vs Rare

- Anemia vs Aplastic Anemia
- Sickle Cell Trait vs Sickle Cell Disease
- Deep Vein Thrombosis vs Fibromuscular Dysplasia



A Few Examples

Hemophilia

- Hemophilia A
- Hemophilia B
- Acquired hemophilia

Clotting Factor Abnormalities

- Plasminogen deficiency
- Factor XI deficiency
- Prothrombin (FII) deficiency
- Alpha 2-antiplasmin deficiency

Hemoglobin Diseases

- Sickle cell disease
- Thalassemia
- Methemoglobinemia

Other

- Myelodysplastic syndromes
- Myelofibrosis
- Gaucher disease

Why Join a Clinical Trial?

Many reasons

- Current treatment not safe
- Current treatment not available
- Current treatment not effective
- Free access to new treatments?
- Advance science
- Other

Are they safe?

- Clinical trials are designed by experts to succeed, not fail (*with the caveat there are no guarantees*)
- Clinical trials have highly regulated and intense safety monitoring.
- Monitoring of side effects more common in clinical trials than in standard care

Talking to a Patient About a Clinical Trial?

Before the meeting

- Is there a clinical trial running?
 - Clinicaltrials.gov, other
- Is the patient likely eligible
 - Inclusion / exclusion criteria
- Location, time commitment?
- What type of clinical trial is it?

Setting up the meeting

- Quiet room with no interruptions
- Allow caregiver/friend/relative to attend
- Pen, paper, laptop to take notes



Talking to a Patient About a Clinical Trial?



Things to do during meeting

- Explain the trial is voluntary
 - Repeat several times
- Explain the disease, current treatment options
 - and then introduce clinical trial
- Use simple terminology
- Listen to the patient (they are in charge)

After the meeting

- Reinforce the notion that the patient has rights
- Schedule a call/meeting with PI
- Determine your role in helping the patient

Common vs Rare Clinical Trials?

	Common Conditions	Rare Conditions
Phase 1	10-100 patients	5-20 patients
Phase 2	100-500	5-40
Phase 3	500-5000	10-500
Inclusion Criteria	Very strict	Strict
Exclusion Criteria	Very strict	Strict
Number of locations	Numerous	Few
Recruiting patients	Easier	More Difficult

How to Research Clinical Trials?

 U.S. National Library of Medicine

ClinicalTrials.gov

[Find Studies](#) ▾ [About Studies](#) ▾ [Submit Studies](#) ▾ [Resources](#) ▾

[Home](#) > Search Results

[Hide Search](#) [Start Over](#)

Condition or disease ⓘ

hemophilia

x

Other terms ⓘ

x

Country ⓘ

⌵

x

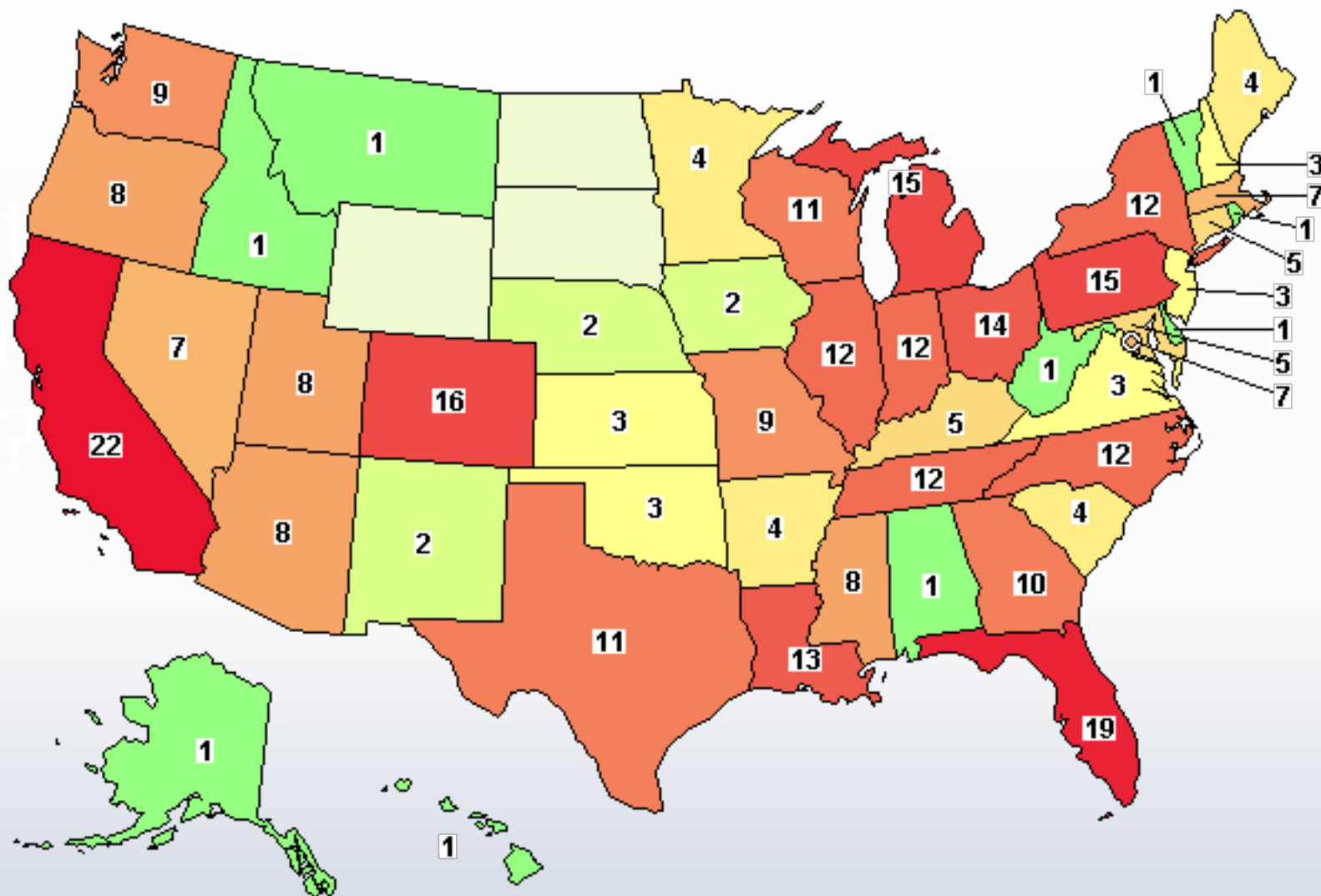
[Search](#) [Advanced Search](#)

41 Studies found for: **Recruiting Studies | hemophilia | United States**

Applied Filters:
☒ **Recruiting**

Search Results (41 trials recruiting)

How many
research
trials?



How to Fine Tune The Search?

The screenshot displays a search interface for clinical trials. At the top, there is a blue 'Search' button and a 'Help' link. Below these are several filter sections:

- Condition or disease:** A text input field containing 'Hemophilia B without Inhibitor' with a clear 'X' button.
- Other terms:** A text input field with a clear 'X' button.
- Study type:** A dropdown menu is open, showing the following options:
 - All Studies
 - ✓ Interventional Studies (Clinical Trials) (highlighted with a blue bar)
 - Observational Studies
 - Patient Registries
 - Expanded Access Studies
- Study Results:** A dropdown menu with a clear 'X' button.
- Status:** A list of checkboxes for study status:
 - ☒ Not yet recruiting
 - ☒ Recruiting
 - ☐ Enrolling by invitation
 - ☐ Active, not recruiting
 - ☐ Suspended
 - ☐ Terminated
 - ☐ Completed
 - ☐ Withdrawn
 - ☐ Unknown status
 - ☐ Available
 - ☐ No longer available
 - ☐ Temporarily not available
 - ☐ Approved for marketing

Search Results (7 trials recruiting)

Study Title	Condition	Intervention	Locations
Fitusiran in Severe Hemophilia A and B Patients w/out Inhibitors	• Hemophilia A • Hemophilia B	Drug: fitusiran Drug: factor VIII or factor IX	44 locations
Fitusiran in Severe Hemophilia A and B Patients with Inhibitors	• Hemophilia A • Hemophilia B	Drug: fitusiran Drug: factor VIII or factor IX	27 locations
AMT-061 in Severe or Moderately Severe Hemophilia B Patients	• Hemophilia B	Genetic: AAV5-hFIXco-Padua	38 locations
Six month lead-in Study to Evaluate Prospective Efficacy and Safety Data of Current FIX Prophylactic Replacement Therapy in Hemophilia B	• Hemophilia A • Hemophilia B	Biological: PF-06838435/fidanacogene elaparvovec	1 location

How to Research Clinical Trials?

 U.S. National Library of Medicine

[Find Studies](#) [About Studies](#) [Submit Studies](#) [Resources](#)

ClinicalTrials.gov

[Home](#) > Search Results

[Hide Search](#) [Start Over](#)

Condition or disease ⓘ

Myelodysplastic Syndromes

X

Other terms ⓘ

X

Country ⓘ

United States

⌵

X

State ⓘ

Minnesota

⌵

X

City ⓘ

Rochester

X

Search

[Advanced Search](#)

10 Studies found for: Recruiting, Not yet recruiting Studies | Myelodysplastic Syndromes | Rochester, Minnesota, U.S.

Also searched for Preleukemia. [See Search Details](#)

Applied Filters: ☒ Recruiting
☒ Not yet recruiting

Search Results (10 trials recruiting)

Study Title	Condition	Intervention	Locations
Clinical Transplant-Related Long-term Outcomes of Alternative Donor Allogeneic Transplantation	• Myelodysplastic syndromes • AML • ALL • 4 others	Donor search prognosis score	8 locations [including Mayo Clinic (PI William Hogan)]
Clinical Transplant-Related Long-term Outcomes of Alternative Donor Allogeneic Transplantation (BMT CTN 1702)	• Myelodysplastic syndromes • Acute leukemia • CML • Lymphoma	Stem cell graft	34 locations (including Mayo Clinic)

Pros and Cons

Pros

- Focused care
- Advancing science
- Free medicine
- Possible treatment

Cons

- Travel expenses
- Time commitment
- Placebo?
- Safety and efficacy?



Case Study

Patient

- 37 yr old male
- Eosinophilic esophagitis
- Current therapies not effective

Pros and Cons

- Patient wants to be enter clinical trial but has some misgivings about:
 - Placebo
 - The impact the trial will have on family
 - Being treated by a different doctor

