

# Data Collection and Analysis from Multiple Research Sites

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Rare Diseases Clinical Research Network\*

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\*Supported by ORD, NCRR

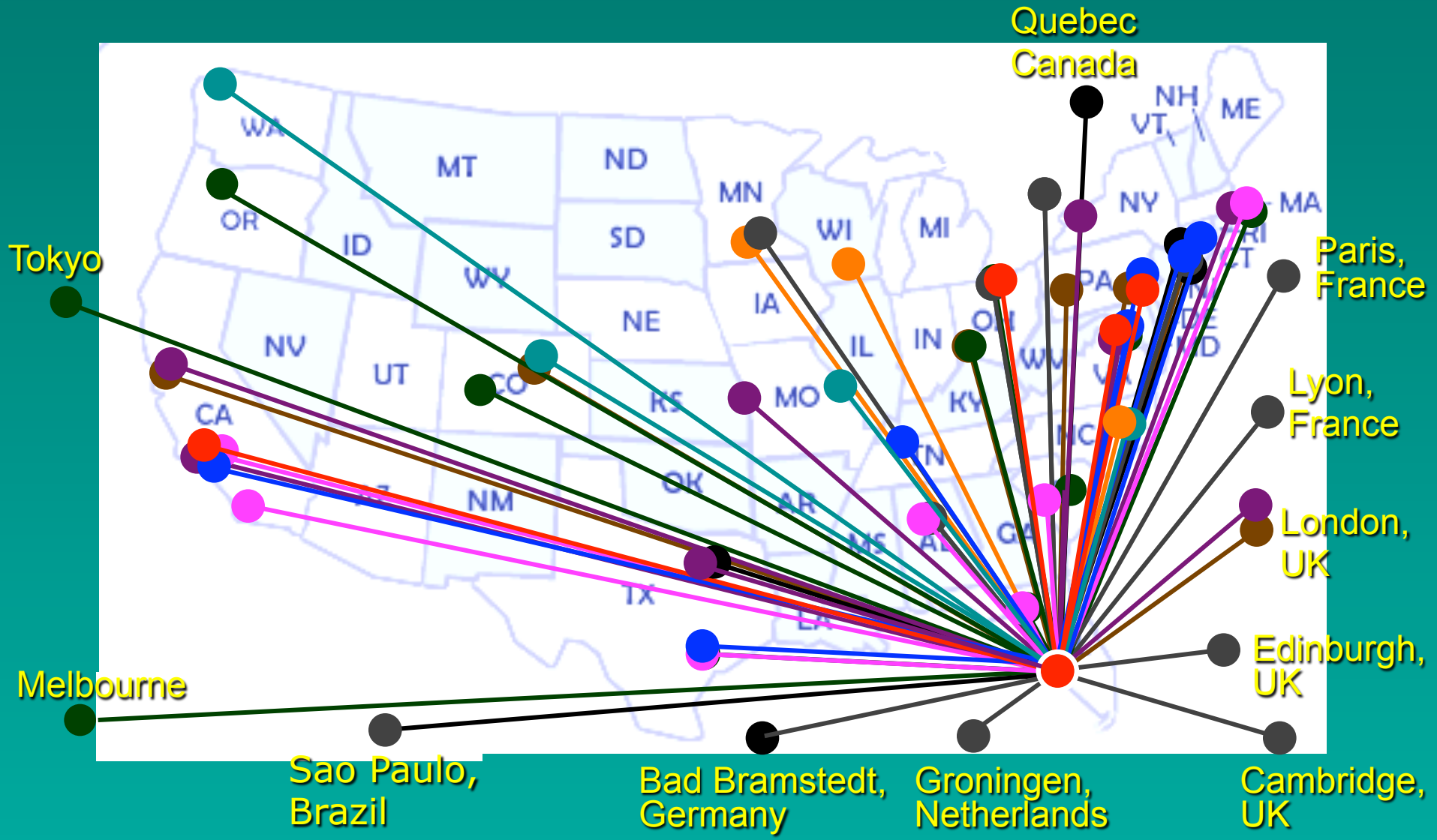
# Rare Diseases Clinical Research Network

## Consortia:

1. Channelopathies
2. Vasculitis
3. Genetic Steroid Disorders
4. Bone Marrow Failure
5. Urea Cycles Disorders
6. Rare Lung Diseases
7. Thrombotic Disorders
8. Genetic Diseases of Mucociliary Clearance
9. Angelman, Rett, PW Syndromes
10. Rare Liver Diseases



# RDCRN Enrollment Sites



# Forty One Rare Diseases are Currently Under Study in the RDCRN

Alpha-1 Antitrypsin Deficiency  
Amegakaryocytic Thrombocytopenic  
Purpura  
Andersen-Tawil Syndrome  
Androgen Receptor Defects  
Angelman's Syndrome  
Antiphospholipid Antibody Syndromes  
Aplastic Anemia  
Apparent Mineralocorticoid Excess  
Arginase Deficiency  
Argininosuccinate Lyase Deficiency  
Argininosuccinate Synthetase Deficiency  
Autoimmune Neutropenia  
Carbamyl Phosphate Synthetase Deficiency  
Catastrophic Antiphospholipid Ab  
Syndrome  
Churg-Strauss Syndrome  
Citrin Deficiency  
Congenital Adrenal Hyperplasia  
Cystic Fibrosis  
Episodic Ataxias  
Giant Cell Arteritis  
Heparin-induced Thrombocytopenia

Hereditary Interstitial Lung Disease  
Large Granular Lymphocyte Leukemia  
Lymphangioleiomyomatosis  
Microscopic Polyangiitis  
Myelodysplastic Syndromes  
N-Acetylglutamate Synthase Deficiency  
Non-dystrophic Myotonic Disorders  
Ornithine Transcarbamylase Deficiency  
Ornithine Translocase Deficiency Syndrome  
Paroxysmal Nocturnal Hemoglobinuria  
Polyarteritis Nodosa  
Prader-Willi Syndrome  
Primary Ciliary Dyskinesia  
Pseudohypoaldosteronism  
Pulmonary Alveolar Proteinosis  
Pure Red Cell Aplasia  
Rett Syndrome  
Takayasu's Arteritis  
Thrombotic Thrombocytopenic Purpura  
Wegener's Granulomatosis

# RDCRN Goals

- ◆ To contribute to the research and treatment of rare diseases by working together to identify biomarkers for disease risk, disease severity and activity, and clinical outcome, while also encouraging development of new approaches to diagnosis, prevention, and treatment.

# DTCC Goals

To provide:

- ◆ A scalable, coordinated, clinical data management system for collection, storage, and analysis of data of RDCRCs,
- ◆ A portal and tools for integration of developed and publicly available datasets for cross-disease data mining at RDCRCs,
- ◆ Web based recruitment and referral tools,
- ◆ A user friendly resource site for the public, research scientists, and clinicians.

# DTCC Roles

## ◆ Protocol Development

# Protocol development tools

- ◆ Data forms development
- ◆ Data collection systems
- ◆ Protocol tracking
- ◆ Automated reporting
- ◆ Interim monitoring
- ◆ Data analysis



# Common Protocol Concepts

- ◆ Common template
- ◆ Model informed consent
- ◆ Common data standards
- ◆ Data sharing
- ◆ External Data Safety and Monitoring Board

# Standards Committee Objectives:

- ◆ Endorse appropriate data representation standards to assure the portability and interoperability of RDCRN research data within and across RDCRN consortia, and with clinical, genomic, and other biomedical data and knowledge.
- ◆ Advocate for the data representation needs of clinical research and rare disease research in current Standards Development Organization (SDO) activities.
- ◆ Encourage the use of endorsed data standards throughout the RDCRN, and, by providing training and education, facilitate data standardization at the source of data collection where possible.

# DTCC Roles

- ◆ Protocol Development
- ◆ Recruitment

Where  
do I need to go

ome! You have reached the home page  
the Rare Diseases Clinical Research  
ork (RDCRN). Each Consortium within the  
ork provides detailed information on  
al rare diseases.

at if I am unsure of which  
consortium to visit?

the list to the right for a disease name.  
you have located it, click on the link for a  
description which will lead you to the  
ct consortium.

y will this consortium be useful  
ne?

can take action! Once you have reached  
correct consortium, you will be able to **join**  
**contact registry** for clinical research  
. You will also find several helpful  
ources that include participating clinical  
er information, self help and advocacy  
o information and other useful links.



## Clinical Research Consortia :

### Urea Cycle Disorders Consortium

N-Acetylglutamate Synthase (NAGS) Deficiency  
Carbamyl Phosphate Synthetase (CPS) Deficiency  
Ornithine Transcarbamylase (OTC) Deficiency  
Argininosuccinate Synthetase Deficiency (Citrullinemia I)  
Citrin Deficiency (Citrullinemia II)  
Argininosuccinate Lyase Deficiency (Argininosuccinic Aciduria)  
Arginase Deficiency (Hyperargininemia)  
Ornithine Translocase Deficiency (HHH) Syndrome

### Angelman, Rett, and Prader-Willi Syndromes Consortium

Angelman's Syndrome  
Rett Syndrome  
Prader-Willi Syndrome

### Consortium for Clinical Investigation of Neurological Channelopathies

Andersen-Tawil Syndrome (Periodic paralysis)  
Episodic Ataxias  
Non-dystrophic Myotonic Disorders

### Bone Marrow Failure Disease Consortium

Aplastic Anemia  
Myelodysplastic Syndromes  
Paroxysmal Nocturnal Hemoglobinuria (PNH)  
Large Granular Lymphocyte (LGL) Leukemia  
Single Lineage Cytopenias:  
- Pure Red Cell Aplasia  
- Megakaryocytic Thrombocytopenic Purpura  
- Autoimmune Neutropenia

### Cholestatic Liver Disease Consortium

PFIC (Progressive Familial Intrahepatic Cholestasis)  
Bile Acid Synthesis Defects  
Alagille Syndrome  
Alpha One Antitrypsin Deficiency

### Vasculitis Clinical Research Consortium

Wegener's Granulomatosis (WG)  
Microscopic Polyangiitis (MPA)  
Churg-Strauss Syndrome (CSS)  
Polyarteritis Nodosa (PAN)  
Takayasu's Arteritis (TAK)  
Giant Cell (Temporal) Arteritis (GCA)

### Rare Genetic Steroid Disorders Consortium

Congenital Adrenal Hyperplasia  
Androgen Receptor Defects  
Apparent Mineralocorticoid Excess (Low Renin Hypertension)

### Rare Thrombotic Diseases Consortium

Antiphospholipid Antibody Syndromes (APS)  
Heparin-induced Thrombocytopenia (HIT)  
Paroxysmal Nocturnal Hemoglobinuria (PNH)  
Catastrophic Antiphospholipid Antibody Syndrome (Thrombotic Storm)  
Thrombotic Thrombocytopenic Purpura (TTP)

### Rare Lung Disease Consortium

Hereditary Interstitial Lung Disease (hILD)  
Lymphangioleiomyomatosis (LAM)  
Pulmonary Alveolar Proteinosis (PAP)  
Alpha-1 Antitrypsin Deficiency (Alpha-1)

### Genetic Diseases of Mucociliary Clearance Consortium

Primary Ciliary Dyskinesia (PCD)  
Cystic Fibrosis  
Pseudohypoaldosteronism (PHA)  
Other Chronic Sinopulmonary Disease



# Patient Contact Registry

Welcome to The Bone Marrow Failure Disease Consortium Web Site - Microsoft Internet Explorer

File Edit View Favorites Tools Help

Back Forward Stop Home Search Favorites RSS Print Mail News

Address <http://www.rarediseasesnetwork.org/bmf/index.htm> Go Links

**What is the BMFDC?**


[Learn More](#)


**Take Action**

**News & Events**

**Participating Clinical Centers**

**Contact Information**

  
**Member Site Login**

  
**Rare Diseases Media Center**

**Welcome!**


**The Bone Marrow Failure Disease Consortium (BMFDC)** is a caring group of doctors, nurses, patients and patient advocates who are dedicated to the education, care, and treatment of patients with rare bone marrow failure diseases. It is our goal to improve the care of patients with aplastic anemia (AA), myelodysplastic syndromes (MDS), pure red cell aplasia (PRCA), paroxysmal nocturnal hemoglobinuria (PNH), and large granular lymphocytic leukemia (LGL).

**We Can Help You**

- Get up-to-date information on your

**Take Action**

Find Patient Support or Advocacy Groups

  
**Join the Contact Registry**

Why **Your** Participation Matters!

What is a Clinical Trial?

Learn About Current Clinical Trials

**BMFDC News**

[Aplastic Anemia & MDS International Foundation held their 2004 Annual Patient & Family Conference in](#)

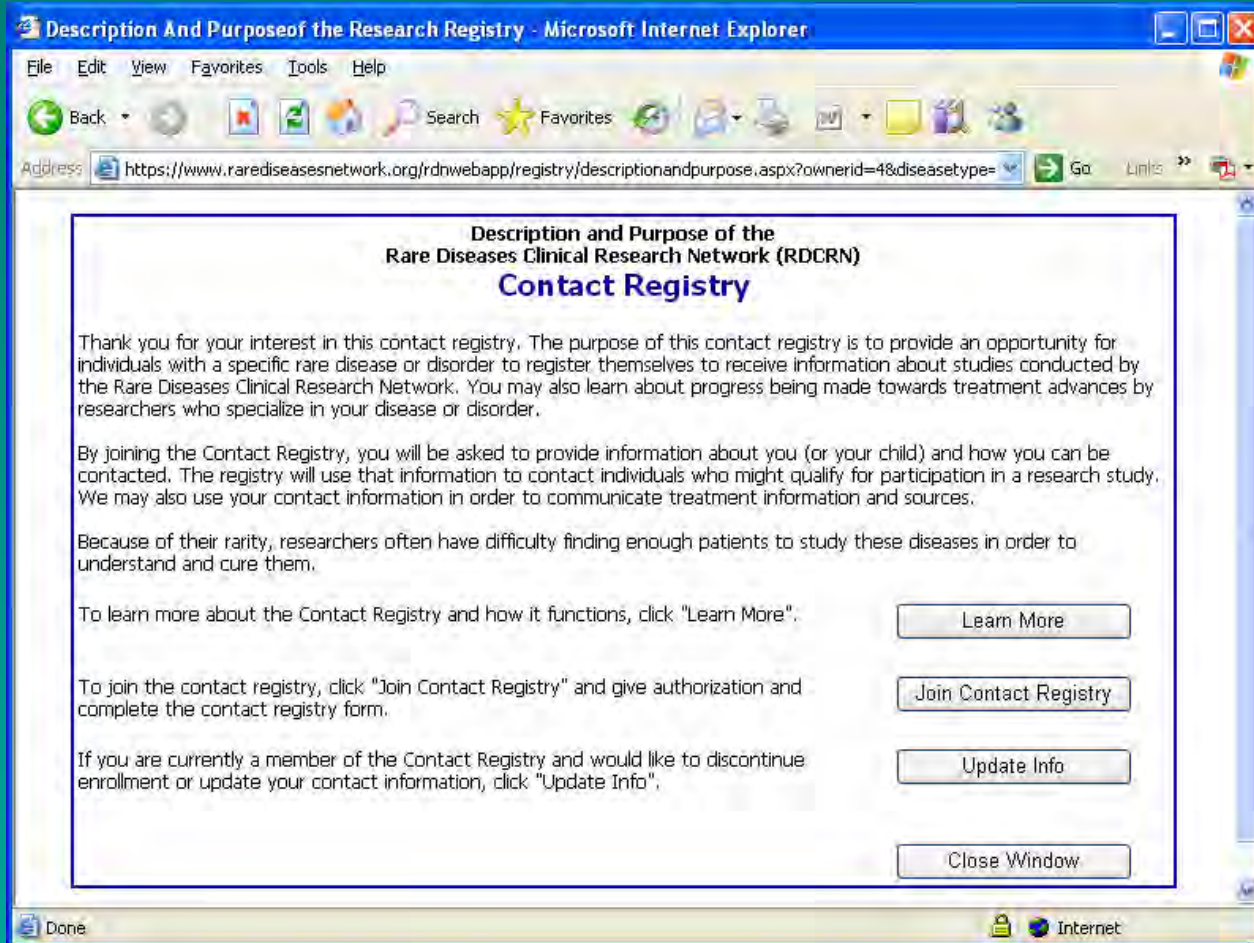
**Learn More**

**Definitions and Explanations** [Learn More](#)

**Diseases:**

- Aplastic Anemia
- Myelodysplastic Syndrome (MDS)
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Large Granular Lymphocyte (LGL) Leukemia
- Single Lineage Cytopenias:
  - + Pure Red Cell Aplasia
  - + Amegakaryocytic Thrombocytopenic Purpura
  - + Autoimmune Neutropenia

<http://www.rarediseasesnetwork.org/bmf/learnmore/index.htm> Internet



## Authorization Agreement

### RDCRN Contact Registry

**Please read the following carefully. The submission of information in the registry will be considered your consent to the following statements.**

#### Authorization Statement for Use and Disclosure of Protected Health Information

The University of South Florida and the Rare Diseases Clinical Research Network Data and Technology Coordinating Center understand that information about you/(your minor child) and your/(your minor child's) health is personal, and we are committed to protecting the privacy of that information. You are granting your authorization before we use your/(your minor child's) protected health information (PHI) for the purpose of providing you (your child) notification of the availability of clinical studies or trials and updates on the results of clinical studies and trials performed within the Rare Diseases Clinical Research Network. This form memorializes your authorization for us to use your PHI for this purpose and helps us make sure that you are properly informed of how this information will be used and/or disclosed.

By agreeing to this document you are permitting the Data and Technology Coordinating Center

I HEREBY GIVE permission to the Rare Diseases Clinical Research Network to use the information I provide to the online patient registry. I understand that if I enter my or my child's contact information, I agree to be contacted about future research studies. I understand that if I do provide my or my child's name or other contact



Registry Form - Microsoft Internet Explorer

File Edit View Favorites Tools Help

Back Forward Stop Reload Home Search Favorites Mail Print Address Book RSS Feeds

Address <https://www.rarediseasesnetwork.org/rdnwebapp/registry/RDCRNRegistrationForm.aspx?OwnerId=4&DiseaseType=> Go Links

## Contact Registry Form

\* Field required to Register

### Patient Information (Person with disorder)

Disorder:  
Aplastic Anemia \*

Date of Diagnosis (if you don't know the entire date, please enter as much as possible)  
Day (DD) Month (MMM) Year (YYYY) \*

Date of Birth (if you don't know the entire date, please enter as much as possible)  
Day (DD) Month (MMM) Year (YYYY) \*

Place of birth:  
County:  
State/Province: Select

Gender: \*  
☐ Male ☐ Female

Done Internet



Registry Form - Microsoft Internet Explorer

File Edit View Favorites Tools Help



Back



Search



Favorites



Address

https://www.rarediseasesnetwork.org/rdnwebapp/registry/RDCRNRegistrationForm.aspx?OwnerId=4&DiseaseTy

Go

Links



Contact Information (Person to be contacted)

First name:

Last name:

Mailing Address:

City:

State/Province:

Country:

Zip/Postal Code:

Primary Phone:

(e.g., 888-555-1212)

Secondary Phone:

(e.g., 888-555-1212)

Email address:

Fax:

(e.g., 888-555-1212)

I am a: \*

☐

Patient

☐

Parent/Guardian

How do you prefer us to contact you?

Done



Internet

# Patient Contact Registry

- ◆ Careful attention to privacy.
- ◆ Disseminate information about new studies.
- ◆ Identify those who are likely to meet eligibility criteria.
- ◆ Provide study related materials.
- ◆ Facilitate contact with clinical programs.

# DTCC Roles

- ◆ Protocol Development
- ◆ Recruitment
- ◆ Data Collection

Consortia Page - Microsoft Internet Explorer

File Edit View Favorites Tools Help


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Search

Favorites

Address <https://www.rarediseasesnetwork.org/rdnwebappdemo/members/ConsortiaPage.aspx?Owner=4&Root=1&Directc> Go Links

- Genetic Steroid Disorders
- Neuro Channelopathies
- Rare Lung Diseases
- Rare Thrombotic Diseases
- Urea Cycle Disorders
- Vasculitis

**Bone Marrow Failure Disease Consortium**



9500 Euclid Avenue,  
R40  
Cleveland, OH 44195  
Fax: 216-636-2498  
Email:  
[maciej@cc.ccf.org](mailto:maciej@cc.ccf.org)


Logo


Add Previous


Consortium Members

Document Navigation



Conference Calls

Grant

Protocols

Protocol Management Tools

5401

Protocol For Data Base And Registry In Idiopathic Bone Marrow Fai...

5402

A Phase II Trial of Tipifarnib (Zarnestra) Efficacy in Large Gran...

5403

A Phase II Trial of Sirolimus (Rapamune?) and Cyclosporine in Pat...

5404

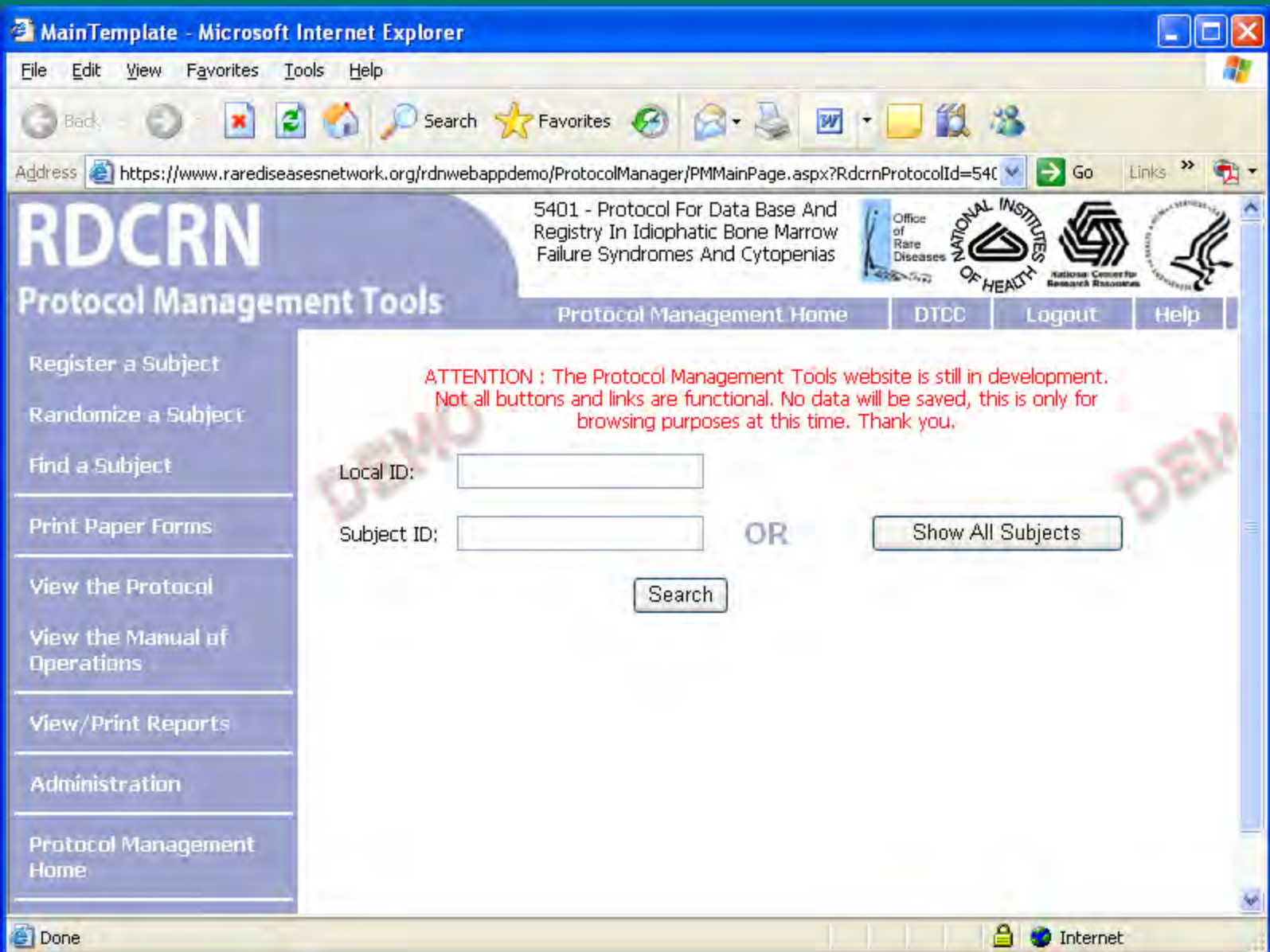
Pilot Trial of SELDI Proteomics in Bone

Coalition of Patient Advocacy Groups (CPAG)

Committees

- Human Subjects Committee
- Longitudinal Studies Committee
- Participant/Community Links Committee
- Publications Committee
- Specimen Tracking Committee
- Standards Committee
- Steering Committee
- Training Committee
- Website Committee

Internet





MainTemplate - Microsoft Internet Explorer

File Edit View Favorites Tools Help

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Address <https://www.rarediseasesnetwork.org/rdnwebappdemo/ProtocolManager/PMMainPage.aspx?RdcnProtocolId=5401> Go Links

# RDCRN

## Protocol Management Tools

5401 - Protocol For Data Base And Registry In Idiopathic Bone Marrow Failure Syndromes And Cytopenias

Office of Rare Diseases NATIONAL INSTITUTES OF HEALTH National Center for Research Resources

Protocol Management Home DTCC Logout Help

Register a Subject  
Randomize a Subject  
Find a Subject  
Print Paper Forms  
View the Protocol  
View the Manual of Operations  
View/Print Reports  
Administration  
Protocol Management Home

ATTENTION : The Protocol Management Tools website is still in development. Not all buttons and links are functional. No data will be saved, this is only for browsing purposes at this time. Thank you.

Local ID:

Subject ID:  OR

Local ID	Subject ID	Study Site	Registration Date	Subject Status
<a href="#">MMM90990</a>	1	The Penn State Cancer Institute	07 Jul 2004	Eligible
<a href="#">BMF01 001</a>	10145	UCLA Department of Hematology and Oncology (BMFDC)	01 Jul 2004	Eligible
<a href="#">BMF01 002</a>	2	UCLA Department of Hematology	20 Jul 2004	Registered

Done Internet

# RDCRN

## Protocol Management Tools

5401 - Protocol For Data Base And Registry In Idiopathic Bone Marrow Failure Syndromes And Cytopenias

[Protocol Management Home](#)[DTCC](#)[Logout](#)[Help](#)[Register a Subject](#)[Randomize a Subject](#)[Find a Subject](#)[Print Paper Forms](#)[View the Protocol](#)[View the Manual of Operations](#)[View/Print Reports](#)[Administration](#)[Protocol Management Home](#)

### Participant's Details

Rare Diseases Clinical Research Network

Protocol # 5401

Subject ID	10145	Date of Registration	01 Jul 2004
Local Subject ID	BMF01 001	Status	Eligible
Site ID	UCLA Department of Hematology and Oncology (BMFDC)		

Timepoint	Event Title	Due Date	Last Modified Date	Event Status
Baseline	Documented Current Medical Illness <a href="#">View/Edit/Print</a>	01 Jul 2004		Tracking
	Past Medical History <a href="#">View/Edit/Print</a>			Tracking
	SF-36 Questionnaire <a href="#">View/Edit/Print</a>			Tracking
	Adverse Event Reporting Form <a href="#">View/Edit/Print</a>			Tracking





CurrentMedicalHistory - Microsoft Internet Explorer

File Edit View Favorites Tools Help

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Address <https://www.rarediseasesnetwork.org/rdnwebappdemo/Forms/04BMFDC/5401/CurrentMedicalHistory.aspx?Event> Go Links »

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Rare Diseases Clinical Research Network Protocol # 5401

Subject ID	10145	Date of Registration	01 Jul 2004
Local Subject ID	BMF01 001	Status	Eligible
Site ID	UCLA Department of Hematology and Oncology (BMFDC)		

---

Documented Current Medical Illness

Date of form collection:

Form completed by:

Historian:

1. Diagnosis per medical record:

Condition	Patient diagnosed with condition?	Date of Diagnosis (dd mon yyyy)
Aplastic Anemia	<input type="radio"/> Yes <input type="radio"/> No	<input type="text"/> <input type="text"/> <input type="text"/>
Myelodysplastic Syndrome (MDS)	<input type="radio"/> Yes <input type="radio"/> No	<input type="text"/> <input type="text"/> <input type="text"/>
Paroxysmal Nocturnal Hemoglobinuria	<input type="radio"/> Yes <input type="radio"/> No	<input type="text"/> <input type="text"/> <input type="text"/>
Large Granular Lymphocytic Leukemia	<input type="radio"/> Yes <input type="radio"/> No	<input type="text"/> <input type="text"/> <input type="text"/>

Done Internet



# Data Collection

- ◆ Web-based protocol management tools :
  - Online data entry
  - Open architecture database systems
  - Electronic information exchange
  - Patient tracking systems
  - Specimen tracking systems
  - Automated reporting

# DTCC Roles

- ◆ Protocol Development
- ◆ Recruitment
- ◆ Data Collection
- ◆ Adverse Event Reporting


# Adverse Event Reporting

http://www.trigr.org/TRIGRDEMO/wcTrigr.ASP?WCI=Main2&WCE=LocalCode[CAN012987] RegistrationNum[1] - Microsoft Internet Explorer p

File Edit View Favorites Tools Help

Back Forward Stop Search Favorites Media Print

Address http://www.trigr.org/TRIGRDEMO/wcTrigr.ASP?WCI=Main2&WCE=LocalCode[CAN012987]%20RegistrationNum[101831] Go Links CKN Dictionary Docs Google

  
**TRIGR**

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[Recruitment](#)  
[Registration](#)  
[Randomization](#)  
[Data - Edit/View](#)  
[- 101831](#)  
[Tracking System](#)  
[Study Management](#)  
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[APTRS Manual](#)  
[Videoconferencing](#)

- Requirements
- User Manual

Success

Adverse Events Editing Successful

**TRIGR**  
Nutritional Prevention of Type 1 Diabetes

Form Number: 121

**Adverse Event Report**

Study Center: CAN01 Local Code: CAN012987  
Registration Code: 101831 Randomization Code: Not Randomized

Complete this form whenever the Study Center learns of an adverse event, either by phone report or subject self-report. Complete additional information reporting forms as indicated.

This form should be completed within one week of learning of the event. If the form is incomplete at the time it is submitted, (e.g., date therapy resumed is not yet known), please revise the online form once the needed information is known.

On this form, each of the "Other" fields and the comment field allow the user to enter up to 250 characters of data (text, numbers, special characters, or spaces). Click on the scroll at the end of each field to see all text.

**Events**

#	* Date Onset	Date Resolved	Date Stopped Formula	Date Resumed Formula
1	12 SEP 2003	15 SEP 2003		
	* Event Code	Other Infection or Allergy Explanation	Other Event Explanation	
	gastroenteritis			
	Location of Treatment	Other Location Explanation	Duration of Treatment	
	emergency room ***			
	Type of Drug Treatment	Other Type of Drug Treatment Explanation		

Additional Comments

Done Internet

✉ **TRIGR Adverse Event Review System: Serious Adverse Event reported. - Message (HTML)**

File Edit View Insert Format Tools Actions Help



From: TRIGRInfo

Sent: Fri 10/3/2003 3:27 PM

To: Krischer, Jeff; Malloy, Jamie; Shanker, Linda K.

Cc:

Subject: TRIGR Adverse Event Review System: Serious Adverse Event reported.

TRIGR

Nutritional Prevention of Type 1 Diabetes

**Adverse Event needs to be assigned for review**

A serious adverse event has been reported (or updated) in the TRIGR data system.

Please login to the TRIGR website ([www.trigr.org/members](http://www.trigr.org/members)) to assign this event for review. Click on the **Adverse Event Review** button on the left menu.

Date of most recent E-mail Notification:	10/3/2003 3:26:42 PM
Event ID:	7
Date Onset:	9/12/2003
Event:	Gastroenteritis
Location of Treatment:	Emergency Room **
Date Event Reported To Study Center:	9/15/2003
Date Online Form Submitted:	9/15/2003


\*\* Indicates serious adverse event.

TRIGR - SplitFramesDemo\_Menu\_ChangePassword.htm - Microsoft Internet Explorer provided by H. Lee Moffitt Cancer Center

File Edit View Favorites Tools Help

Back Forward Stop Home Search Favorites Media Print

Address http://www.trigr.org/TRIGRDEMO/wcTRIGR.ASP?wCI=Welcomedemo&WCE=Login Go Links CKN Dictionary Docs



# TRIGR

**Data Management**

- Recruitment
- Registration
- Randomization
- Data - Edit/View
- Tracking System

**Study Management**

- Download Forms
- Protocol
- Manual of Operations
- Adverse Event Review

**Reports**

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**News**

**Directory**

- Find a Person
- Committee List

**DMU Contact Info**

**Change Password**

**Log Out**

**Home**

**Instructional Materials**

- APRTS Manual
- Videoconferencing
  - Requirements
  - User Manual

## TRIGR

Nutritional Prevention of Type 1 Diabetes

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### Adverse Event Review System

[Click here for instructions](#)

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To be assigned

Event ID	Subject ID/ Local Code	Date Onset	Date Resolved	Event	Location of Treatment	Type and Duration of Drug Treatment	Date Event Recorded	Status	Action
7	101831/ CAN012987	12/Sep/2003	15/Sep/2003	Gastroenteritis	Emergency room		03/Oct/2003	To be assigned	Jamie Malloy <input type="button" value="OK"/>

Done
Internet

✉ **TRIGR Adverse Event Review System: Serious Adverse Event reported. - Message (HTML)**

File Edit View Insert Format Tools Actions Help



From: TRIGRInfo

Sent: Fri 10/3/2003 3:31 PM

To: Unknown; Malloy, Jamie; Shanker, Linda K.

Cc:

Subject: TRIGR Adverse Event Review System: Serious Adverse Event reported.

TRIGR

Nutritional Prevention of Type 1 Diabetes

**Adverse Event needs review**

A serious adverse event has been reported (or updated) in the TRIGR data system.

Please login to the TRIGR website ([www.trigr.org/members](http://www.trigr.org/members)) to review this event. Click on the **Adverse Event Review** button on the left menu.

Date of most recent E-mail Notification:	10/3/2003 3:30:42 PM
Event ID:	7
Date Onset:	9/12/2003
Event:	Gastroenteritis
Location of Treatment:	Emergency Room **
Date Event Reported To Study Center:	9/15/2003
Date Online Form Submitted:	9/15/2003

\*\* Indicates serious adverse event.



**Data Management**

Recruitment

Registration

Randomization

Data - Edit/View

- 101831

Tracking System

**Study Management**

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Videoconferencing

• Requirements

• User Manual

**TRIGR**Nutritional Prevention of Type 1 Diabetes  
Internal Safety Monitoring Group**Adverse Event Review Form**

Event ID		Subject Status		Event Status	
7		Registered Only		Waiting for review	
Event Information					
Date Onset	12/Sep/2003	Date Event Recorded	03/Oct/2003		
Date Resolved	15/Sep/2003	Event	Gastroenteritis		
Date Stopped Formula		Location	Emergency room		
Date Resumed Formula		Treatment	None(< 1 week)		
Date of Birth	12/Jan/2003	Age of Onset	34 week(s) 5 day(s)		
Additional Comments		Infant treated for diarrhea			
Member Comments					
- No comments -					

[Click here to view all other adverse events for this subject.](#)

**After reviewing the above event(s) please complete and submit this form:**

Was event caused by participation in study? ☐ No ☐ Yes ☐ Likely Yes ☒ Likely No

Do you recommend study change? ☒ No ☐ Yes

If yes, what is your recommendation?

May need to stop Study Formula for test trial.

Additional comments (maximum 750 characters):

Do you request further ISMG discussion about this event? ☐ No ☒ Yes

Submit

Print Form

The application is running in Demonstration Mode. Do not enter real study data.

## TRIGR

### Nutritional Prevention of Type 1 Diabetes

#### Adverse Event Summary

<a href="#">Event ID</a>	<a href="#">Subject Status</a>	<a href="#">Event Status</a>
8	Registered Only	Unknown

Event Information			
Date Onset	12/Jun/2003	Date Event Recorded	03/Oct/2003
Date Resolved	18/Jun/2003	Event	Middle ear infection
Date Stopped Formula		Location	Office visit
Date Resumed Formula		Treatment	Antibiotics(1 - 2 weeks)
Date of Birth	12/Jan/2003	Age of Onset	21 week(s) 4 day(s)
Additional Comments			

#### Review Information

- No review -

#### Member Comments

- No comments -

<a href="#">Event ID</a>	<a href="#">Subject Status</a>	<a href="#">Event Status</a>
9	Registered Only	Unknown

Event Information			
Date Onset	18/Apr/2003	Date Event Recorded	03/Oct/2003
Date Resolved	19/Apr/2003	Event	Asthma, other allergy
Date Stopped Formula		Location	Office visit
Date Resumed Formula		Treatment	Inhaled corticosteroids(< 1 week)
Date of Birth	12/Jan/2003	Age of Onset	13 week(s) 5 day(s)
Additional Comments			

#### Review Information

- No review -

#### Member Comments

- No comments -



**Data Management**

Recruitment

Registration

Randomization

Data - Edit/View

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**Study Management**

Download Forms

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**Instructional Materials**

APRTRS Manual

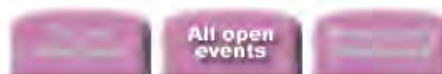
Videoconferencing

• Requirements

• User Manual

**TRIGR**

Nutritional Prevention of Type 1 Diabetes

**Adverse Event Review System**[Click here for instructions](#)**Waiting for review (0)**

Event ID	Date Event Recorded	Event	Location of Treatment	Assigned To	Date Event Assigned	Status	Action
----------	---------------------	-------	-----------------------	-------------	---------------------	--------	--------

No events waiting for review.

**Up for discussion (1)**

Event ID	Date Event Recorded	Event	Location of Treatment	Discussion	Status	Action
7	03/Oct/2003	Gastroenteritis	Emergency room	0 members have commented	Up for discussion	<input type="button" value="Discuss"/> <input type="button" value="Close"/>

**Reviewed (0)**

Event ID	Date Event Recorded	Event	Location of Treatment	Reviewed By	Review Date	Review Result	Status	Action
----------	---------------------	-------	-----------------------	-------------	-------------	---------------	--------	--------

No open reviewed events.

**TRIGR****Data Management**

Recruitment

Registration

Randomization

Data - Edit/View

Tracking System

**Study Management**

Download Forms

Protocol

Manual of Operations

Adverse Event Review

**Reports**

- General
- Local

**News****Directory**

- Find a Person
- Committee List

DMU Contact Info

Change Password

Log Out

Home

**Instructional Materials**

APRTRS Manual

Videoconferencing

- Requirements
- User Manual

The application is running in Demonstration Mode. Do not enter real study data.

**TRIGR**Nutritional Prevention of Type 1 Diabetes  
Internal Safety Monitoring Group**Adverse Event Comment Form**

Event ID	Subject Status	Event Status	
7	Registered Only	Up for discussion	
<b>Event Information</b>			
Date Onset	12/Sep/2003	Date Event Recorded	03/Oct/2003
Date Resolved	15/Sep/2003	Event	Gastroenteritis
Date Stopped Formula		Location	Emergency room
Date Resumed Formula		Treatment	None(< 1 week)
Date of Birth	12/Jan/2003	Age of Onset	34 week(s) 5 day(s)
Additional Comments	Infant treated for diarrhea		
<b>Review Information</b>			
Study related?	Likely No	Recommend change?	No
Recommendations	May need to stop Study Formula for test trial.	Reviewer	Jamie Malloy
Comments			
<b>Member Comments</b>			
- No comments -			

[Click here to view all other adverse events for this subject.](#)

Add your comments here (maximum 750 characters):

I disagree.



**Data Management**

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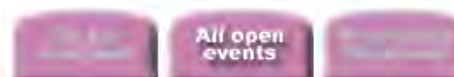
Videoconferencing

• Requirements

• User Manual

**Success** Event successfully closed.**TRIGR**

Nutritional Prevention of Type 1 Diabetes

**Adverse Event Review System**[Click here for instructions](#)**Waiting for review (0)**

Event ID	Date Event Recorded	Event	Location of Treatment	Assigned To	Date Event Assigned	Status	Action
----------	---------------------	-------	-----------------------	-------------	---------------------	--------	--------

No events waiting for review.

**Up for discussion (0)**

Event ID	Date Event Recorded	Event	Location of Treatment	Discussion	Status	Action
----------	---------------------	-------	-----------------------	------------	--------	--------

No events up for discussion.

**Reviewed (0)**

Event ID	Date Event Recorded	Event	Location of Treatment	Reviewed By	Review Date	Review Result	Status	Action
----------	---------------------	-------	-----------------------	-------------	-------------	---------------	--------	--------

No open reviewed events.

# Adverse Event Reporting

- ◆ Electronic notification & review.
- ◆ Central administration.
- ◆ Role of medical monitor.
- ◆ Automated reporting.

# DTCC Roles

- ◆ Protocol Development
- ◆ Recruitment
- ◆ Data Collection
- ◆ Adverse Event Reporting
- ◆ Dissemination of Research Results

## What is the BMFDC?

[Learn More](#)

[Take Action](#)

[News & Events](#)

[Participating Clinical Centers](#)

[Contact Information](#)



## Welcome!

### The Bone Marrow Failure Disease Consortium (BMFDC)

is a caring group of doctors, nurses, patients and patient advocates who are dedicated to the education, care, and treatment of patients with rare bone marrow failure diseases. It is our goal to improve the care of patients with aplastic anemia (AA), myelodysplastic syndromes (MDS), pure red cell aplasia (PRCA), paroxysmal nocturnal hemoglobinuria (PNH), and large granular lymphocytic leukemia (LGL).

### We Can Help You

- Get up-to-date information on your

## Take Action

Find Patient Support or Advocacy Groups



Why **Your** Participation Matters!

What is a Clinical Trial?

Learn About Current Clinical Trials

## BMFDC News

[Aplastic Anemia & MDS International Foundation held their 2004 Annual Patient & Family Conference in](#)

## Learn More

### Definitions and Explanations of Diseases:

- Aplastic Anemia
- Myelodysplastic Syndrome (MDS)
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Large Granular Lymphocyte (LGL) Leukemia
- Single Lineage Cytopenias:
  - + Pure Red Cell Aplasia
  - + Amegakaryocytic Thrombocytopenic Purpura
  - + Autoimmune Neutropenia







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Online education on rare diseases research for healthcare professionals and patients

**Presentations** Presentations

**Conferences** Note: The presentations require Microsoft Windows Media Player to view. 

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Title	Speaker	Date ▼	Credit
<a href="#">Pathogenesis of LGL Leukemia</a>	Thomas P. Loughran Jr. M.D.	12/13/2002	
<a href="#">Apoptosis and Drug Resistance in Hematologic Malignancies</a>	Thomas P. Loughran Jr. M.D. Terry H. Landowski Ph.D.	2/4/2000	

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# Rare Diseases Media Center

Online education on rare diseases research for healthcare professionals and patients

## Pathogenesis of LGL Leukemia

**Presentations**

**Conferences**

**Webcasts**

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**Speaker:**

**Thomas P. Loughran Jr., M.D.**  
Professor of Medicine  
H. Lee Moffitt Cancer Center & Research Institute  
Tampa, FL

**Original Date:** 12/13/2002


**Date of Expiration:** 12/13/2004


**Length:** 52 minutes

**Objectives:**

- Examine the classification of LGL leukemia and become familiar with clinic presentations of these diseases.
- Understand how abnormal Fas/Fas ligand interactions contribute to disease pathogenesis.

**Speaker Disclosure:** The speaker has indicated that he receives Grant Support from Hisamitsu Pharmaceuticals.



 [Tell a Colleague](#)

E-mail address:

Done Internet

# Common threads

- ◆ Multi-center, multi-national, multi-disciplinary studies.
- ◆ Web-based data collection.
- ◆ Management of large, diverse data bases.
- ◆ Clinical Trials.
- ◆ Epidemiology studies.

# Common threads (continued)

- ◆ Live webcasts
- ◆ Internet-based videoconferencing
- ◆ Video libraries
- ◆ Electronic data exchange with labs and remote sites
- ◆ Interactive voice response systems for registration/randomization

# Summary

- ◆ Collectively, these systems facilitate research in rare diseases by
  - transcending geographic boundaries,
  - providing a comprehensive informatics environment for the conduct of studies in many different clinical settings and
  - promoting standards that enhance the value of the accumulating data for future research.

# Acknowledgement

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