Utilizing Public Health Surveillance to Monitor Adverse Outcomes of Blood Product Therapy

Coordinating Center for Health Promotion
National Center on Birth Defects and Developmental Disabilities

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May, 2005

Prevention Program

- **Division of Hereditary Blood Disorders**

Mission:

- To reduce or prevent complications of hemophilia and other bleeding & clotting disorders & thalassemia
- Mandated by Congress

Prevention Program

- Approximately 18,000 people in the U.S. have hemophilia
- Treatment for bleeding consists of infusions of biopharmaceutical products made from blood
- Potential risk of infectious disease transmission including hepatitis and HIV
- CDC has established a public health surveillance system for product safety

Prevention Program

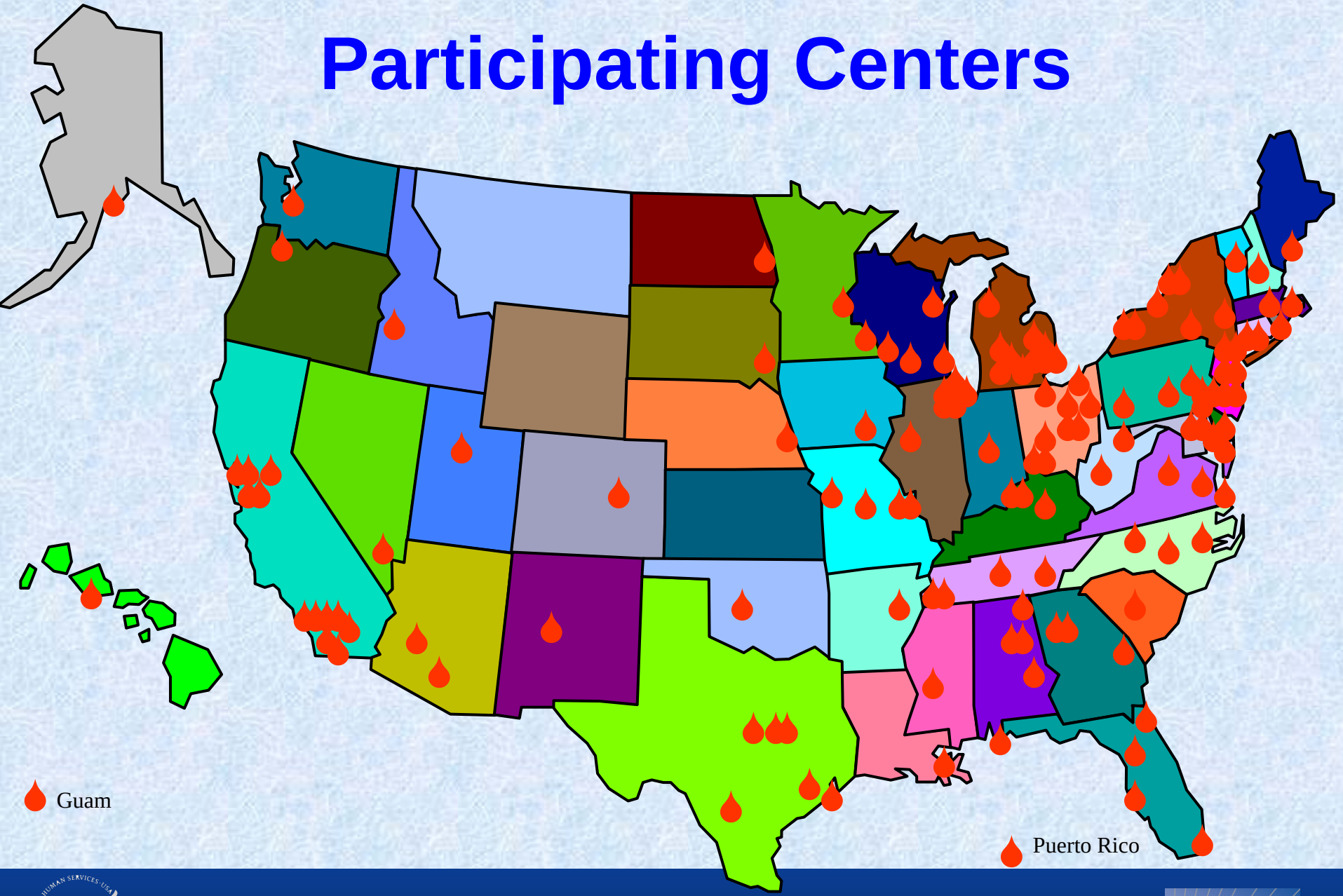
- **Target Priorities**
  - Blood product safety
  - Joint Disease
  - Women with bleeding disorders
  - Detection of hereditary abnormalities associated with bleeding & clotting disorders

Prevention Program

CDC/HTC Cooperative Agreement

- Participate in blood safety monitoring and surveillance efforts
- Collaborate with lay organizations to deliver consistent prevention messages
- Maintain a prevention evaluation network to assess the efficacy of these prevention services

Participating Centers



Bleeding Disorder Surveillance

- Universal Data Collection System (UDC)
  - Monitor blood safety among the recipients of blood products
  - Monitor extent and progression of joint disease
  - Identify issues for further study

Universal Data Collection
Study Design

- National protocol approved by CDC and local human Investigational Review Boards.
- Standardized data collection annually using tools designed with input from experts
- Patients donate a plasma specimen annually
  - Portion is stored in CDC Serum Bank
  - Tested for hepatitis and HIV
- New infections are investigated for link with product

Universal Data Collection

Data Elements

- Data collected by treatment center staff
  - Demographic (Date of birth, race, sex)
  - Clinical (Hemophilia type, severity)
  - Treatment (Bleeding and infusion frequency)
  - All blood products used in past year
  - Infectious disease (Liver disease, joint infections)
  - Impact of joint disease on daily living
  - Joint range-of-motion measures

UDC Enrollment

- Since May, 1998, 16,217 persons with bleeding disorders have been enrolled
- There have been 40,041 UDC visits
- The overall national refusal rate is 7.6%
- Approximately 40% of UDC annual visit data are being submitted electronically using a clinical software tool

Updated May 2005

Blood Safety Monitoring

- No new infections with HIV or hepatitis due to blood products among UDC participants
- Many patients at risk for hepatitis A infection are receiving vaccinations - particularly important to vaccinate those who are hepatitis C infected
- Provides reassurance to the bleeding disorder community of product safety
- Serum bank established for future use
- Special investigations of Parvovirus B19 and WNV have been performed

Blood Safety Studies

- Evidence from community for blood-borne transmission of WNV
- Susceptible to viral inactivation steps
- West Nile Virus testing among patients with hemophilia with visits held during previous mosquito seasons
- To date, there is no evidence of WNV transmission through blood products

Thalassemia Program

- Monitor blood safety and reduce the rate of complications among persons with thalassemia on monthly blood transfusions
- Since June, 2004 we have received data and plasma samples on 200 patients
- Storing and testing plasma samples
- Developing data collection tools
  - Transfusion reactions
  - Complications of iron overload

Inhibitor Pilot Study

- Some hemophilia patients develop antibodies to factor products (inhibitor)
- Patients with inhibitors are at increased risk of poor outcomes
- Risk for inhibitors may be related to the factor product used
- Piloting a protocol for post-marketing surveillance of treatment products

Key Features

- Clinical centers with dedicated staff and access to the patient population
- Data collection tools must:
  - Collect minimum amount of needed data
  - Seek to collect data that are easily available
- Perform regular and frequent data analyses
- Make the results available and useful
- Patient understanding and acceptance is key to successful study recruitment

UDC Working Group

- Provides expert input to CDC on all aspects of UDC including data selection and interpretation
- Monthly conference calls and annual meetings

- Tom Abshire, MD
- Steve Arkin, MD
- Randy Curtis, MBA
- John Drake, RC
- Nancy Duffy, RN
- Angela Forsyth, PT
- Sue Geraghty, RN
- W. Keith Hoots, MD
- Nigel Key, MD
- Ed Kuebler, MSW
- Barbara Konkle, MD
- Roshni Kulkarni, MD

Surveillance Reporting

- Routine surveillance reports
- Published articles
- National, regional, HTC reports on web
- HTC specific annual report
  - Summary of patient characteristics
  - Comparison by HTC, Region, Nation
  - Provides a global perspective

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