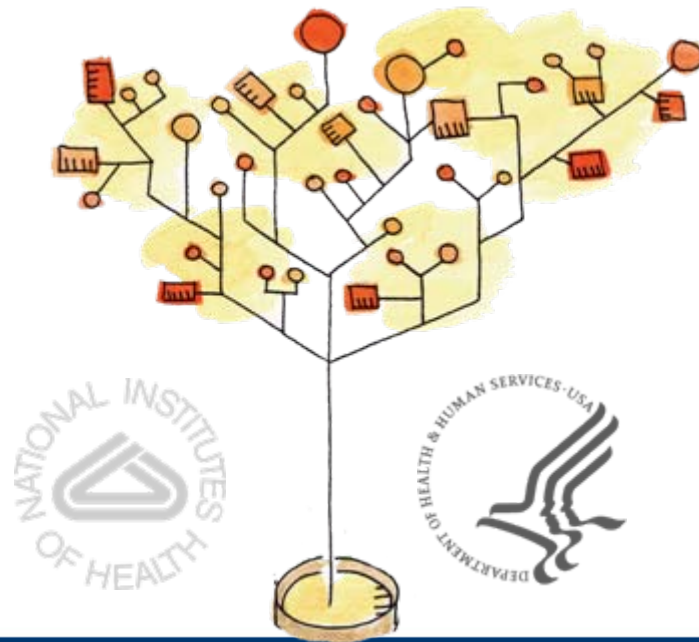


Rare and Neglected Diseases Pieces of the Puzzle

Bonny Harbinger, Ph.D., J.D.
Deputy Director
Office of Technology Transfer
National Institutes of Health
U.S. Department of Health & Human Services

ICORD -2008



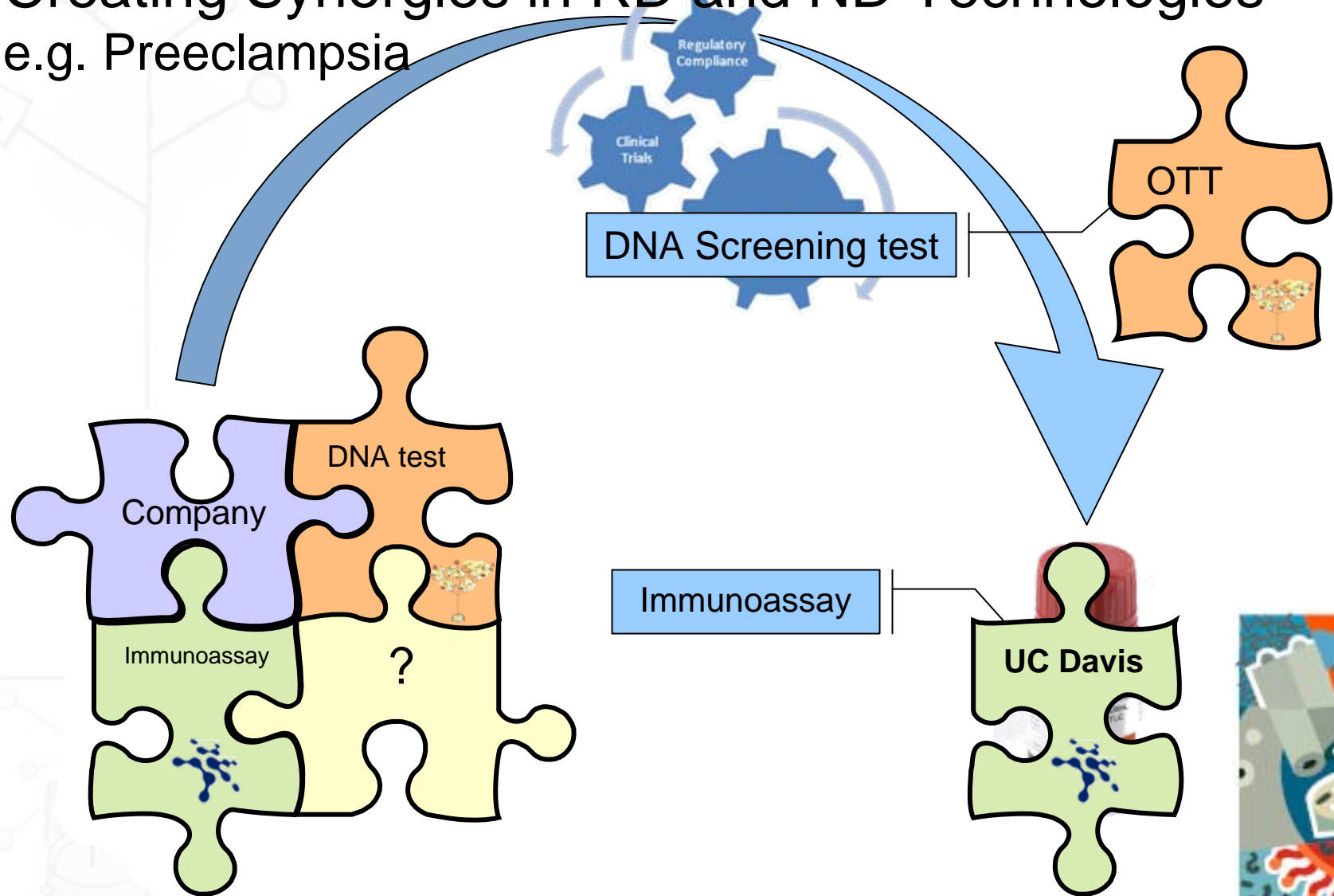
NIH Intramural Research Program

- 6,000 intramural scientists
- 2,000 active projects
- Basic & clinical research discoveries
- Collaborations with industry & academia
- 224 Rare disease related technologies
- ~ 1,400 technologies available for licensing
- ~200 products developed to date
(24 vaccines and therapeutics)



Creating Synergies in RD and ND Technologies

e.g. Preeclampsia



Rare

- **Webs**

- **Host:**

 - **OTT**

- **Partic**

 - **OTT**

- **Visito**

 - **Licen**

Participants

Baystate Health System
Beth Israel Deaconess Medical Center
Boston Biomedical Research Institute
Boston University
Brigham and Women's Hospital
Massachusetts General Hospital
Children's Hospital
Dana-Farber Cancer Institute
Harvard University
Joslin Research
Massachusetts Eye and Ear Infirmary
McLean Hospital
National Jewish Medical & Research Center
St. Jude Children's Research Hospital
The CBR Institute for Biomedical Research
The University of British Columbia
Tufts University
University of Arizona
University of Arkansas for Medical Sciences
University of California, Davis
University of California, Los Angeles
University of Massachusetts
University of Rochester Medical Center
Yeda R&D Co. Ltd.

Marketing



Rare Diseases and Conditions

Technologies Available for Licensing from NIH/FDA and Non-Profit Institutions

Technologies related to rare diseases or conditions available for commercial licensing are listed under the related disease or condition. You can search disease names alphabetically or by specific name. Note that, at present, there may not be a listed invention for every disease or condition. Please continue to revisit the site as it will be updated periodically. The diseases and conditions on the site are those designated as such by the NIH Office of Rare Diseases and are defined as having a prevalence of fewer than 200,000 affected individuals in the US. For background information on a specific disease or condition, please visit the [NIH Office of Rare Diseases Website](#) or click on the [\[Define\]](#) button next to the disease name.

If you are a non-profit institution and would like to have your technology included in this list, please [Click Here](#).

[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](#) | [\[1-9\]](#) | [All](#)

- ◆ [Acoustic neuroma \[Define\]](#)
- ◆ [Acute lymphoblastic leukemia, Childhood \[Define\]](#)
- ◆ [Acute respiratory distress syndrome \[Define\]](#)
- ◆ [Adenocarcinoma of lung \[Define\]](#)
- ◆ [Adrenal cancer \[Define\]](#)
- ◆ [Albinism \[Define\]](#)
- ◆ [Alpha 1-antitrypsin deficiency \[Define\]](#)
- ◆ [Alzheimer disease type 1 \[Define\]](#)
- ◆ [Amyloidosis \[Define\]](#)
- ◆ [Anaplastic large cell lymphoma \[Define\]](#)
- ◆ [Anthrax \[Define\]](#)
- ◆ [Ataxia telangiectasia \[Define\]](#)
- ◆ [Autoimmune myocarditis \[Define\]](#)
- ◆ [Actinomycetales infection \[Define\]](#)
- ◆ [Acute myelogenous leukemia \[Define\]](#)
- ◆ [Addison's disease \[Define\]](#)
- ◆ [Adenosine deaminase deficiency \[Define\]](#)
- ◆ [Adrenocortical carcinoma \[Define\]](#)
- ◆ [Alkaptonuria \[Define\]](#)
- ◆ [Alpha-Thalassemia \[Define\]](#)
- ◆ [Alzheimer disease type 2 \[Define\]](#)
- ◆ [Amyotrophic lateral sclerosis \[Define\]](#)
- ◆ [Aneuploidy \[Define\]](#)
- ◆ [Aplastic anemia \[Define\]](#)
- ◆ [Autoimmune hepatitis \[Define\]](#)



Rare Diseases and Conditions

Technologies Available for Licensing from NIH/FDA

Preeclampsia

Determination of AM-Binding Proteins and the Association of Adrenomedullin (AM) Therewith

The present invention provides methods for the isolation, identification, and purification of adrenomedullin (AM)- binding proteins. Methods for utilizing the purified AM-binding proteins, or functional portions thereof, to diagnose, treat, and monitor AM-related diseases are described. A second aspect of this technology discloses the identification and isolation of a novel complex between AM and a specific AM-binding protein 1 (AMBP-1), design... [More ...](#)

The Many Roles Of Adrenomedullin In Human Pathology And Physiology

Adrenomedullin (AM) is an a-amidated 52-amino acid peptide that shows slight similarity to calcitonin gene-related peptide (CGRP). The effects of AM and its fragments in the cardiovascular system have been widely studied. Endothelial cells secrete the peptide that acts on specific receptors present in the vascular smooth muscle cells and other contractile cells. Some diuretic functions have been also described. In the respiratory system, two majo... [More ...](#)

Method Evolved for Recognition of Thrombophilia (MERT): Clinical Predictive Genetic Test for Venous Thrombosis

Venous thrombosis (VT) is one of the leading causes of mortality and morbidity resulting in approximately 300,000 hospitalizations and 50,000 fatalities per year in the United States with an incidence of 141 per 100,000 African-Americans, 104 per 100,000 Caucasians and 21 per 100,000 Asian/Pacific Islanders. However, it is an avoidable disease if effective preventive measures such as early thromboprophylaxis are instituted.

It is hig... [More ...](#)

Technologies Available for Licensing from Non-Profit Institutions

University of California, Davis

[DIAGNOSTIC IMMUNOASSAY FOR HYPERTENSION, ARDS, CARDIOVASCULAR DISEASE, AND LIPID DISORDERS](#)

Boston Biomedical Research Institute, Watertown, MA

[Leptin Peptide Antagonists for cancer, endometriosis, preeclampsia and fertility regulation](#)

Children's Hospital, Boston, MA

[Methods And Compositions For Treatment Of Preeclampsia And Other Pregnancy Complications.](#)

[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](#) [\[1-9\]](#) [All](#)

- > [Overview](#)
- > [Pagets disease](#)
- > [Pancreatic islet cell tumors](#)
- > [Papillitis](#)
- > [Parainfluenza virus type 3](#)
- > [Paraplegia](#)
- > [Parvovirus antenatal infection](#)
- > [Pasteurella multocida](#)
- > [Pemphigus](#)
- > [Pemphigus vulgaris](#)
- > [Pendred syndrome](#)
- > [Peripheral neuropathy](#)
- > [Peripheral T-cell lymphoma](#)
- > [Pheochromocytoma](#)
- > [Pick disease of t' brain](#)
- > [Plaque](#)
- > [Pneumoconiosis](#)
- > [Poliomyelitis](#)
- > [Polyarthriti](#)
- > [Polyomavirus Infections](#)
- > [Precocious pube](#)
- > [Preeclampsia](#)
- > [Premature aqini](#)



Technologies Available for Licensing from NIH/FDA

Preeclampsia

Determination of AM-Binding Proteins and the Ass

The present invention provides methods for the isolation, identification, and characterization of AM-binding proteins, or functional portions thereof. A second aspect of this technology discloses the identification of AM-binding protein 1 (AMBP-1), design... [More ...](#)

The Many Roles Of Adrenomedullin In Human Path

Adrenomedullin (AM) is an a-amidated 52-amino acid peptide that has been shown to have effects of AM and its fragments in the cardiovascular system have been described. In the respiratory system, two major roles of AM and its fragments have been described. In the respiratory system, two major roles of AM and its fragments have been described. In the respiratory system, two major roles of AM and its fragments have been described. [More ...](#)

Method Evolved for Recognition of Thrombophilia and Thrombosis

Venous thrombosis (VT) is one of the leading causes of mortality in the United States with an incidence of 21 per 100,000 Asian/Pacific Islanders. However, it is an avoidable condition if thromboprophylaxis are instituted.

It is hig... [More ...](#)

Technologies Available for Licensing from Non-Profit Organizations

University of California, Davis

DIAGNOSTIC IMMUNOASSAY FOR HYPERTENSION, ARDS, CARDIOVASCULAR DISEASE, AND LIPID DISORDERS

Boston Biomedical Research Institute, [link: DIAGNOSTIC IMMUNOASSAY FOR HYPERTENSION, ARDS, CARDIOVASCULAR DIS...](#)

[Leptin Peptide Antagonists for cancer, endometriosis, preeclampsia and fertility regulation](#)

Children's Hospital, Boston, MA

[Methods And Compositions For Treatment Of Preeclampsia And Other Pregnancy Complications.](#)

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 0 1 2 3 4 5 6 7 8 9

DIAGNOSTIC IMMUNOASSAY FOR HYPERTENSION, ARDS, CARDIOVASCULAR DISEASE, AND LIPI...

File Edit View History Bookmarks Tools Help

http://.../rare diseases" patent life

University of California
OTT
OFFICE OF
TECHNOLOGY
TRANSFER
Office of the President

Non-Confidential Description

DIAGNOSTIC IMMUNOASSAY FOR HYPERTENSION, ARDS, CARDIOVASCULAR DISEASE, AND LIPID DISORDERS

Polyunsaturated fatty acids are important precursors for many regulatory lipids, including lipids involved in the regulation of blood pressure and lipid metabolism. Thus, they are implicated in a number of important disorders such as hypertension, eclampsia, ARDS, cardiovascular disease, and disorders in lipid metabolism. Some of these disorders, like pre-eclampsia, eclampsia, and pregnancy-induced hypertension (PIH) are potentially severe complications in human gestation. Full-blown eclampsia, though rare, has very

Done

- > [Pneumoconiosis](#)
- > [Poliomyelitis](#)
- > [Polyarthritis](#)
- > [Polyomavirus Infections](#)
- > [Precocious puberty](#)
- [Preeclampsia](#)**



Neglected Diseases

SCIENCE. IDEAS. BREAKTHROUGHS.

Neglected Diseases

Technologies Available for Licensing from NIH/FDA and Non-Profit Institutions

Global health organizations define neglected diseases as those that are prevalent among impoverished and marginalized populations in the developing world. Tropical environments, poor conditions, and limited access to health care contribute to the risk and spread of these diseases.

Technologies related to neglected diseases available for commercial licensing can be viewed by clicking on the disease of interest. The list is a compilation of diseases recognized as neglected by the World Health Organization. Note that, at present, there may not be a listed invention for every disease. Please continue to revisit the site as it will be updated periodically. For additional information on a specific disease, click on the [Define] link.

If you are a non-profit institution and would like to have your technology included in this list, please [Click Here](#).

Bacterial Infections

- ♦ [Buruli ulcer \(Mycobacterium ulcerans\)](#) [Define]
- ♦ [Cholera \(Vibrio cholerae\)](#) [Define]
- ♦ [Leprosy \(Mycobacterium leprae\)](#) [Define]
- ♦ [Trachoma \(Chlamydia trachomatis\)](#) [Define]
- ♦ [Tuberculosis \(Mycobacterium tuberculosis\)](#) [Define]
- ♦ [Yaws \(Treponema pertenue\)](#) [Define]

Helminthic Infections

- ♦ [Dracunculiasis \[Guinea-worm disease\] \(Dracunculus medinensis\)](#) [Define]
- ♦ [Fascioliasis \(Fasciola sp.\)](#) [Define]
- ♦ [Hookworm \(Ancylostoma duodenale, Necator americanus\)](#) [Define]
- ♦ [Lymphatic filariasis \[Elephantiasis\] \(Wuchereria bancrofti, Brugia malayi\)](#)
- ♦ [Onchocerciasis \[River blindness\] \(Onchocerca volvulus\)](#) [Define]
- ♦ [Roundworm \(Ascaris lumbricoides\)](#) [Define]
- ♦ [Schistosomiasis \[Bilharzia or snail fever\] \(Schistosoma sp.\)](#) [Define]
- ♦ [Whipworm \(Trichuris trichiura\)](#) [Define]

Protozoan Infections

- ♦ [African Trypanosomiasis \[Sleeping sickness\] \(Trypanosoma brucei\)](#) [Define]
- ♦ [Chagas disease \(Trypanosoma cruzi\)](#) [Define]
- ♦ [Leishmaniasis \(Leishmania sp.\)](#) [Define]
- ♦ [Malaria \(Plasmodium sp.\)](#) [Define]

Viral Infections

- ♦ [Dengue \(Flaviviridae\)](#) [Define]
- ♦ [Rotavirus \(Reoviridae\)](#) [Define]

OTT RD Statistics

- Number of RD on site: 290
- Number of External Institutes: 24
- Number of External Technologies: 182



Thank you!

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