

## Genetic Diseases of Children: Advancing Research & Care

March 7, 2011 - March 9, 2011

Sheraton New York Hotel & Towers, New York, NY

### Agenda

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There are no additional charges for any of the meals or sessions.

This agenda is subject to change

Last updated: September, 8, 2010

#### Theme Areas

I

Reaching An Early Diagnosis

II

Sharing Family Experiences

III

Optimizing Care And Coordination Of Services

IV

Meeting Children's Need

V

Accelerating Research

VI

All

### Monday March 7, 2011

#### Conference Opening

VI

4:00 pm - Conference Registration Opens

VI

6:00 pm - Reception

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VI

7:00 pm - Dinner

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**Tuesday March 8, 2011**

**Plenary A**

8:30 am - 10:00 am

VI

Diagnosis, Research and Care for Rare Diseases: A Conference Overview

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**Break**

10:00 am - 10:30 am

VI

Break

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**Session 1**

10:30 am - 11:15 am

I

Clinical Benefits And Limitations Of Newborn Screening

II

Day To Day Challenges We Face

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III

Integrating Family History And Genomic Information Into Medical Care

IV

Early Intervention Services: Diet And Medication; Medical Foods

V

Advocacy Groups As Research Organizations

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### Session 2

11:30 am - 12:15 pm

I

Should Newborns Be Screened For A Disorder If: I. The Value And/Or Necessity Of Treatment Is Uncertain? II: No Treatment Is Available?

II

Impact Of Our Diagnosis On Our Family

III

Integrating Natural History And Treatment History Knowledge Into Medical Care

IV

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Complementary And Alternative Therapies

V

Enrolling In Natural History Studies And Clinical Trials

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### Lunch

12:15 pm - 1:30 pm

VI

Lunch

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### Plenary B

1:30 pm - 3:00 pm

VI

Whole Genome/Exome Sequencing For Diagnosis: Are We There Yet?

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### Session 3

3:15 pm - 4:00 pm

I

What Will Newborn Screening Look Like In 2020?

II

Meeting The Psychosocial Needs Of Families And Other Caregivers

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III

Optimizing Care: Lessons Learned

IV

Physical Therapy And Occupational Therapy

V

Recruiting And Training Young Investigators: Program  
Directors' And Funders' Perspectives

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### Session 4

4:15 pm - 5:00 pm

I

Complexities Of Genome Analysis For Genetic Diagnosis

II

Importance Of Family Support Programs And Social Networks

III

Best Practices For Achieving Optimized Levels Of Care

IV

Education And Artistic Expression

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V

Recruiting And Training Young Investigators: Young Investigators' And Affected Families Perspectives

**Wednesday March 9, 2011**

**Plenary C**

8:30 am - 10:00 am

VI

NIH And FDA Initiatives For New Therapies

**Break**

10:00 am - 10:30 am

VI

Break

**Session 5**

10:30 am - 11:15 am

I

Carrier Screening: What To Screen For? What To Test For? When?

II

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Navigating The Healthcare System; Having A Patient-Centered Medical Home

III

Federal Programs For Children And Families; AND Impact Of Health Care Reform

IV

Long Term Care Plans; Importance Of Genetic Counseling

V

Therapy Development, Lessons Learned. Part I. Clinical Trial Readiness, Trial Designs For Small Populations

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### **Session 6**

11:30 am - 12:15 pm

I

The Undiagnosed Disease Program At NIH AND Will There Always Be A Diagnostic Odyssey?

II

Getting Reliable Information AND What Constitutes An Expert?

III

State And Local Programs For Children And Families

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IV

Transition Between Pediatric And Adult Care; Insurance Coverage

V

Therapy Development, Lessons Learned. Part II: Novel Strategies And Relationships

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### **Lunch Session**

12:15 pm - 1:30 pm

VI

Keynote Speaker: Jim Kelly, Founder Hunter's Hope Foundation

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### **Plenary D**

1:30 pm - 3:00 pm

VI

Back To The Future: The Road Ahead

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### **Session 7**

3:15 pm - 4:00 p.m.

II

Ask The Experts: Psychologists, Social Workers, And Genetic Counselors



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V

Therapeutic Advances: Molecular Approaches

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**Session 8**

4:15 pm - 5:00 pm

II

Ask The Experts: Researchers, Caregivers And Clinicians

V

Therapeutic Advances: Cellular Approaches