Science & Society Symposium for Families: Advocacy, Awareness & Fundraising

The Importance of DS Research & How DS Organizations and Families Can Advocate for Government Funding

Michelle Sie Whitten, Co-Founder, President & CEO

Saturday, June 8, 2019 • T21RS Biennial Conference, Barcelona
What We Will Cover Today

- **Welcome & Speaker Intros**
  - Megan Bomgaars, actress from the 4-time Emmy award winning Born this Way hit series, and entrepreneur - *Self-Advocates with Down Syndrome Make it Happen!*
  - Keith Smith, Crnic Institute Human Trisome Project Program Manager, Brother and Katherine Waugh, Crnic Institute Postdoctoral Fellow, Sister-in-law, Down Syndrome World Magazine contributor - *Scientists with Family Members with Down Syndrome - Things to Consider Before Participating in Research*
  - Manuel Velázquez, Vice President, DOWN ESPAÑA and Michelle Sie Whitten, - *Engaging the Press, Self-Advocates & Celebrities to Raise Awareness & Funds*

- **Importance of Down Syndrome Research & Medical Care**
  - Global’s Mission - to significantly improve the lives of people with Down syndrome through research & medical care
  - Importance of Research & Medical Care

- **How Down Syndrome Organizations and Families Can Advocate for Government Funding**

- **Q&A/Thank YOU**
An Intro to Michelle

I am:

- **first and foremost a Mom! with...**
  - one awesome husband, Tom
  - two terrific kids, Sophia and Patrick
  - Sophia happens to have Down syndrome

- **first generation Italian-Chinese American**
  - My mother is from Italy and my father is from China - they met in NJ of course!
  - I am one of five kids and I was lucky enough to attend Tufts, Harvard and Peking Universities

- **a cable baby**
  - My dad worked in aerospace then became a cable TV pioneer - the father of “digital compression,” the founder of Starz/Encore channels and more
  - I worked in Asia in cable TV for 15 years!
  - We lobbied foreign governments for participation in cable TV and programming ownership

- **the founder and Pres/CEO of Global**
  - Global has a focus on research & medical care - including government advocacy for this area
The Catalyst to Start Global
A life-threatening disparity of funding at the NIH

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The >300,000 Americans with trisomy 21 may hold the key to major medical conditions.

- Cancer
- Heart disease
  - Coronary Artery Disease
  - Atherosclerosis
  - Hypertension
  - Angiopathies

- Alzheimer’s
- Autoimmunity
  - Autism
  - Epilepsy, Infantile Spasms
  - Congenital Heart Defects
  - Autoimmune Disorders: Celiac, Hashimoto’s, T1D, Vitiligo, Alopecia Areata, etc
  - Vision Problems
  - Hearing Problems
  - Intestinal Atresia
  - Sleep Apnea

The Catalyst to Start Global
A Radically Different Disease Spectrum

T21RS Barcelona, Science & Symposium for Families
June 8, 2019 - Government Advocacy for DS Research
The Global Down Syndrome Foundation is part of a network of affiliate organizations that work closely together on a daily basis to deliver on our mission - Significantly improve the lives of people with Down syndrome through Research, Medical Care, Education & Advocacy:

- **Global**: fundraising, outreach, advocacy for Global and four Global Affiliates.
- **Sie Center**: over 1,700 unique patients from 28 states and 10 countries with 8 clinics (2 first in-kind)
- **Crnic Institute**: over 200 scientists working to elongate life and improve health outcomes for people with Down syndrome.
- **RMADC**: Leukine and other important breakthroughs.
- **Adult Clinic**: Pilot phase until June 2019. Goal is to have a world-class feeder for the Sie Center.
Global Down Syndrome Foundation: IMPORTANCE OF RESEARCH
How we are ‘More Alike’ than different

How we are very different!

- Highly Predisposed - Alzheimer’s Disease, Autoimmune Disorders, Certain Leukemias, Autism, etc.
- Highly Protected - Solid Tumor (Cancers), Heart Attack and certain types of Strokes

What we are LUCKY to have!

- Healthcare Guidelines for Children - American Association of Pediatrics (AAP)
- Pediatric Specialty Clinics - 60+ Pediatric Centers across the US and growing

What we desperately need!

- Healthcare Guidelines for Adults - thanks to our guideline authors! Drs. George Capone, Brian Chicoine, Dennis McGuire, Kent McKelvey, Barry Martin, Peter Bulova, and Moya Peterson, PhD APRN, Bryn Gelaro, LSW, Michael Wells, BS
- Adult Specialty Clinics - only a handful in the U.S.
- RESEARCH and FUNDING - fueling all of the above!
**Excellent, Appropriate Medical Care is Important!**
- It increases the length of life
- It improves the quality of life
- It allows people with Down syndrome to reach their true potential

**Scientific Research Directly Influences Medical Care!**
- Research can help us create excellent guidelines for medical care
- Research can help us solve the worst medical ailments that are more common in people with Down syndrome - Alzheimer’s Disease, Immune System Disorders, certain Leukemias
- **No current guidelines for adults with Down syndrome (Drs. William I. Cohen and David S. Smith); 2001 “Health Supervision for Children with Down Syndrome”; current ones are a “must” and provide a great check-list (Drs. Marilyn J. Bull, William I. Cohen, Nancy Rozien)**

**People with Down Syndrome have a Different Disease Spectrum!**
Basic Research
- generates new ideas, principles, and theories, which may not be immediately utilized but nonetheless form the basis of progress and development in different fields. Basic research rarely helps practitioners directly with their everyday concerns; nevertheless, it stimulates new ways of thinking that have the potential to revolutionize and dramatically improve how practitioners deal with a problem in the future.

Translational Research
- applies findings from basic science to enhance human health and well-being. In a medical research context, it aims to "translate" findings in fundamental research into medical practice and meaningful health outcomes. Translational research implements a "bench-to-bedside", from laboratory experiments through clinical trials to point-of-care patient applications, model, harnessing knowledge from basic sciences to produce new drugs, devices, and treatment options for patients.

Wikipedia Search!
Clinical Research

- is a branch of healthcare science that determines the safety and effectiveness (efficacy) of medications, devices, diagnostic products and treatment regimes intended for human use. These may be used for prevention, treatment, diagnosis or for relieving symptoms of a disease. Clinical research is different from clinical practice. In clinical practice established treatments are used, while in clinical research evidence is collected to establish a treatment.

Clinical Trials

- are experiments or observations done in clinical research. Such prospective biomedical or behavioral research studies on human participants are designed to answer specific questions about biomedical or behavioral interventions, including new treatments (such as novel vaccines, drugs, dietary choices, dietary supplements, and medical devices) and known interventions that warrant further study and comparison. Clinical trials generate data on safety and efficacy.

Wikipedia Search!
Natural History Research

➢ The natural history of disease is the course a disease takes in individual people from its pathological onset ("inception") until its eventual resolution through complete recovery or death. ... Natural history of disease is one of the major elements of descriptive epidemiology.

Epidemiology

➢ a branch of medical science that deals with the incidence, distribution, and control of disease in a population.

Review of the Literature

➢ is a text of a scholarly paper...they are secondary sources, and do not report new or original experimental work. Most often associated with academic-oriented literature, such reviews are found in academic journals. A narrow-scope literature review may be included as part of a peer-reviewed journal article presenting new research, serving to...provide context for the reader.

Wikipedia Search!
The Healthy Pipeline

Translational Research Model

Basic Research
- Genomics
- Proteomics
- Metabolomics

Mechanisms Research
- Drugs
- Markers
- Devices
- Treatments

Clinical Research
- Clinical Trials
- Epidemiology

Clinical Care
- Education
- Strategy
- Feasibility
- Products
- EB-Guidelines

Outcome
- Populations
- Quality of Care

Bioinformatics

Medical Informatics

Brain & Spinal Injury Website
Spinal Muscular Atrophy (SMA)

- SMA is a neuromuscular disorder, the leading genetic cause of death among children under age two.
- SMA is the result of mutations in the Survival of Motor Neuron 1 (SMN1) gene. Low levels of SMN protein affect nerve cells of the spinal cord. Babies progressively lose their ability to move, breathe and swallow.

Prof Adrian Krainer, Ph.D., Cold Spring Harbor Laboratory

- Using a splicing process to edit RNA, Dr. Krainer has successfully corrected the splicing defect that causes SMA in systems first in test tubes, then in cells taken from SMA patients and grown in the lab, and then in genetically engineered mouse models of SMA.
- In 2009, Dr. Krainer and colleagues identified a compound that stimulates survival of motor neuron (SMN) protein production by altering RNA splicing.
- In December 2016, the FDA approved nusinersen (Spinraza), the first drug approved to treat children (including newborns) and adults with SMA.
- In 2017, Spinraza was approved in Europe, Canada, Japan, Brazil & Switzerland.
A real life example!
Global Down Syndrome Foundation:
OUR STRATEGY - OUR WORK
The Strategy

- Science
  - Therapeutic leverage - Extreme predispositions (Alzheimer’s disease, autoimmune disorders) and protections (solid tumors, certain strokes & heart attack) BUT first and foremost people with Down syndrome.
  - REBUILD the pipeline with excellent science

- Medical Care
  - Establish a world-class pediatric and adult clinics providing excellent care, publishing best practice standards, and a bridging clinical and basic research.

- Lobbying
  - TRANSCENDS Global/Crnic Institute
  - Congressional Champions
  - Self-Advocates and Families
  - Report Language every year
  - Deep knowledge of the DS science and medical care nationally
  - Hire the best lobbying/consulting firms in the biomedical field

- Outreach & Education
  - Organize conferences, workshops, and health & wellness programs that help people with Down syndrome directly in Colorado, the US and internationally

The Sie Family Research Summit, from left to right: First row: Betsy Hoffman, John J. Sie, Tom Cech, Leslie Leinwand, Michelle Sie Whitten, John Sladek; Second row: David Braddock, Hans Lehrach, Mike Yarus, Larry Gold, Enid Ablowitz, Roger Reeves; Third row: Bill Strauss, Kathleleen Gardiner, Mary Lou Oster-Granite; Top row: Michael Allshouse, David Solenger
Global - Proud of Our Accomplishments
Crnic Institute for Down Syndrome Anschutz Medical Campus

Dr. Joaquín Espinosa, Crnic Institute ED
- World-renowned cancer scientist; Howard Hughes Medical Investigator (HHMI)
- Ph.D. from the University of Buenos Aires in Argentina; post-doctoral training at The Salk Institute for Biological Studies in La Jolla, CA
- Professor in the Department of Pharmacology, Co-Leader of the Molecular Oncology Program, Founding Director of the Functional Genomics Facility, University of Colorado Denver School of Medicine

Dr. Huntington Potter, Lead AD Researcher
- Discovered the mechanistic relationship between Alzheimer’s disease and Down syndrome; PROMISING LEUKINE TRIALS UNDERWAY
- Kurt N. and Edith von Kaulla Memorial Professor of Neurology, Director, RMADC, Vice Chair of Research Department of Neurology University of Colorado
- Graduated with a AB, MA and PhD from Harvard in Physics, Chemistry, Biochemistry & Molecular Biology; Professor at Harvard Department of Neurobiology from 1985 - 1998
- Professor and Eric Pfeiffer Chair of Research on Alzheimer’s disease and head of the NIH funded Alzheimer’s Center University of South Florida SOM 1998 - 2012
Global - Proud of Our Accomplishments

40 Labs, 200+ Scientists Working on DS Research

Alzheimer’s Disease

Bettcher
Dell’Acqua
Hoeffer
Kennedy
Potter
Xue

Cognition, Autism and Brain Function

Chen
Maclean
Jones
Maier
Mueller
Santos
Shaikh
Stitzel

Immunology

Lenz
Hsieh
Shen
Spritz
Sullivan
Yeager

Stem Cells and Development

Bilousova
Klymkowsky
Link
Old
Olwin
Pearson

Advanced Genetics and Genomics

Blumenthal
D’Alessandro
Dowell
Johnson
Liu
O’Connor

Leukemia

DeGregori
Espinosa
Yi

19

T21RS Barcelona, Science & Symposium for Families

June 8, 2019 - Government Advocacy for DS Research
The largest, most detailed study of the human population with Down syndrome

Employing the most advanced technology in precision personalized medicine to understand the multiple effects of Down syndrome

Goal - 1,000 people with Down syndrome and 500 typical “controls”; As of today, more than 500 volunteers have participated

Creating a biobank of samples from people with Down syndrome

- Will be able to find similarities and cohorts where you previously only had a single case study

Accessible for researchers and clinicians who have limited or no access to people with Down syndrome

Improving the lines of communication between practitioners and researchers
Everywhere we look, it is clear that trisomy 21 causes increased Interferon signaling.

In typical people the Interferon pathway is lit up when fighting a virus or infection. In people with Down syndrome it is lit up 24/7...a huge taxation on the immune system.
GOVERNMENT ADVOCACY
Low Government Funding - Despite being the leading cause of developmental delay in the U.S. and the world, Down syndrome is one of the least funded genetic conditions by the NIH and has been since 2001.

Precipitous decline in funding - From 2001 to 2006, NIH funding for Down syndrome research plummeted from $29 million to $14 million despite significant growth of the NIH budget.

Parity - From 2001 to 2017, Down syndrome funding would have been $744 million - more than double the actual $356 million - had this research been funded in parity with the NIH budget.

Comparables - Annual NIH research funding for Down syndrome is 2x to 45x less per capita compared to diseases with similar prevalence (e.g. Multiple Sclerosis) or chromosomal conditions and developmental disabilities (e.g. Fragile X or Autism).
Global’s Government Advocacy Goals

Legislation and Funding

1. Dramatically increase research funding at the National Institutes of Health (NIH) benefitting people with Down syndrome - this can also help millions without the condition

2. Move Down syndrome research to a Trans-NIH initiative under the Office of the Director
There is a population explosion of people with Down syndrome in the U.S. that requires dramatically more funding not less

- Population - is estimated between 250,000 and 430,000
- Live Births - have increased to 1 in 691 today from 1 in 1,000 in 2002
- Lifespan - has more than doubled to 60 years from 28 years in the 1980s
- A Mini Population Explosion - will happen over the next several decades due to increased live births and lifespan
- Societal Trends - include a small but growing number of people with Down syndrome participating in college programs, choosing to get married, and living independently or semi-independently

There is a “eugenics framework” in countries like Iceland and Denmark...

THERAPEUTIC LEVERAGE - by studying people with Down syndrome we can possibly cure or help cure major life-threatening diseases
What is NIH?

- The National Institutes of Health (NIH), a part of the U.S. Department of Health and Human Services, is the nation’s medical research agency – making important discoveries that improve health and save lives.

- The National Institutes of Health is made up of 27 different components called Institutes and Centers. Each has its own specific research agenda, often focusing on particular diseases or body systems. All but three of these components receive their funding directly from Congress, and administrate their own budgets. NIH leadership plays an active role in shaping the agency's research planning, activities, and outlook.

- The Office of the Director is the central office, responsible for setting policy for NIH and for planning, managing, and coordinating the programs and activities of all the NIH components.
Down syndrome offers research opportunities across many of the 27 Institutes and Centers

- **Lower risk of heart disease and higher risk of congenital heart defects**
- **Significantly elevated risk for early-onset Alzheimer's**
- **Protected from solid tumor cancers and greater risk for blood cancers**
- **Much lower risk of stroke**
- **Much greater risk of diabetes**
- **~30% experience mental illnesses such as depression or OCD**
- **>60% have vision problems**
- **High rate of dermatological disorders and arthritis**
- **Unique pattern of immune dysregulation**
- **Large pediatric population and unique pattern of development**
- **Most prevalent chromosomal disorder**

**NIH**

- **National Heart, Lung, and Blood Institute**
- **National Institute on Aging**
- **National Institute of Neurological Disorders and Stroke**
- **National Cancer Institute**
- **National Institute of Deafness and Other Communication Disorders**
- **National Institute of Diabetes and Digestive and Kidney Diseases**
- **National Institute of Arthritis and Musculoskeletal and Skin Diseases**
- **National Institute of Allergy and Infectious Diseases**
- **National Institute of Mental Health**
- **National Institute on Drug Abuse**
- **National Institute of Dental and Craniofacial Research**
- **Fogarty International Center**
- **National Center for Advancing Translational Sciences**
- **National Center for Complementary and Integrative Health**
- **National Institute of Biomedical Imaging and Bioengineering**
- **Center for Scientific Review**
- **Center for Information Technology**
KEY TO SUCCESS IS PEOPLE and LOTS OF HARD WORK

- **Self-Advocates and Families**
  - Directly and through members/orgs
- **Scientists & Clinicians**
  - Directly and through DSMIG, T21RS
- **Congressional Champions**
  - Congresswoman Cathy McMorris Rodgers (R-WA)
  - Congressman Tom Cole (R-OK)
  - Congresswoman Rosa DeLauro (D-CT)
  - Congresswoman Cheri Bustos (D-IL)
  - Senators Roy Blunt (R-MO) & Patty Murray (D-WA), and so many more...
- **NIH Champions**
  - Dr. Francis Collins, Director
  - Dr. Lawrence Tabak, Deputy Director NIH
  - Dr. Diana Bianchi, Director of NICHD and her team (Dr. Melissa Parisi, Lisa Kaeser, Sujata Bardhan)
  - Dr. Richard Hodes and his team at NIA (Dr. Laurie Ryan, Erika Tarver)
  - Dr. Gary Gibbons, Director of NIHLB
  - More every year...
NIH & Congressional Meetings
- Global was the first to highlight the co-occurrence of Alzheimer’s disease in the Down syndrome (DS) population AND diseases people with DS are protected against
- 2004/2006 Dr. Elias A. Zerhouni (then director of the NIH) provided Global with the Disparity Funding #s for Down syndrome research at the NIH; Linda Crnic organizes a “State of the Union” for DS Research in DC

REPORT LANGUAGE & BRIEFINGS
- Since 2005 powerful report language inquiring into the disparity of NIH funding for Down syndrome research AND highlighting therapeutic leverage
- Briefings on DS and Alzheimer’s disease, Cancer, Research & Medical Care

FIRST DOWN SYNDROME CONFERENCE AT NIH
- (Dec 2010) NIH and Global co-hosted the first Down Syndrome Conference in NIH history that led to the creation of the DS-Connect patient registry
- Global’s investment in research was the catalyst for Patrick Kennedy and Pete Sessions to start the Congressional Down Syndrome Caucus (Task Force)
- Organized two national workshops with the national Alzheimer’s Association
As Global Champions for Down Syndrome Research we...

- Applaud the efforts of National Institutes of Health (NIH) to encourage research for Down syndrome by establishing the first Down syndrome patient research registry called DS-Connect®.
- Believe that Down syndrome, the leading cause of developmental delay in the world, deserves more research funding to explain why people with Down syndrome have a different ‘disease spectrum’ whereby they are highly protected from some diseases like certain cancers, stroke, and heart attack and are highly susceptible to other diseases such as Alzheimer’s disease and immune system disorders.
- Urge the White House, Congress, and NIH to make Down syndrome research funding a national priority so that we can discover therapies and cures to major diseases that could be life-saving to people with Down syndrome and millions of others suffering from the same life-threatening diseases.

SIGN OUR PETITION OF SUPPORT
FOR MORE FUNDING FOR DOWN SYNDROME RESEARCH

ADD YOUR NAME:

- First Name
- Last Name
- Email
- Street
- Zip Code
- Country
- I am signing because:

KEEP ME UPDATED ABOUT DOWN SYNDROME NEWS
ADD YOUR NAME

Global’s Research Funding Petition
Garnered 7,333 signatures of 5,000 goal
INCLUDE (IN)vestigation of Co-occurring conditions across the Lifespan to Understand Down syndrome (E) Project Research Plan www.nih.gov/include-project

“Down syndrome. The agreement directs the NIH Director to develop a new trans-NIH initiative - involving, at a minimum, NICHD, NIA, and NCI - to study trisomy 21, with the aim of yielding scientific discoveries to improve the health and neurodevelopment of individuals with Down syndrome and typical individuals at risk for Alzheimer's disease, cancer, cardiovascular disease, immune system dysregulation, and autism, among others. This initiative shall bring together research results that will be available to academic researchers, nonprofit organizations, and industry researchers. Funding for this trans-NIH initiative will supplement, not supplant, existing NIH funding levels for Down syndrome research.”
On October 25, 2017, Global hosted the first-ever Congressional hearing on Down syndrome

“Down Syndrome: Update on the State of the Science & Potential for Discoveries Across Other Major Diseases”

Call to action for increased funding from the National Institutes of Health on Down syndrome research
Frank Stephens testifies Before Congress ... over 100M views resulting in a historic 71% increase of NIH’s Down syndrome research budget from $35mil in FY2017 • to $60 mil in FY2018 • to $77mil in FY2019
Videos from Congressional Hearing

- **WATCH CLIP: 2017 CHAIRMAN TOM COLE (R -OK)**
  Congressman from Oklahoma, Ranking Member Labor, Health and Human Services, Education, and Related Agencies

- **WATCH CLIP: 2017 RANKING MEMBER ROSA DELAURO (D-CT)**
  Congresswoman from Connecticut, Chairwoman Labor, Health and Human Services, Education, and Related Agencies

- **WATCH CLIP: Frank Stephens**
  Quincy Jones Advocate, Global Down Syndrome Foundation

- **WATCH CLIP: Michelle Sie Whitten**
  President & CEO Global Down Syndrome Foundation
  Executive Director Anna & John J. Sie Foundation

- **WATCH CLIP: Joaquín Espinosa, PhD**
  Professor of Pharmacology, University of Colorado Denver School of Medicine, Co-Lead, Molecular Oncology Program and University of Colorado Cancer Center, Executive Director, Linda Crnic Institute for Down Syndrome

- **WATCH CLIP: Cathy McMorris Rodgers (R-WA)**
  Congresswoman from Washington, House Republican Conference Chair

- **WATCH CLIP: Bill Mobley, PhD**
  Executive Director, Down Syndrome Center for Research and Treatment, and Florence Riford Chair of Alzheimer’s Disease Research, UC San Diego

- **Watch the whole hearing!**
### NIH Historic Increases in Down Syndrome Research

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People with Down syndrome, families and Down syndrome organizations CAN SUCCESSFULLY ADVOCATE FOR DOWN SYNDROME RESEARCH GOVERNMENT FUNDING
How You Can Help  
Do your homework!

- Identify your NIH equivalent - Instituto de Salud Carlos III - ISCIII? Subdirección General de Proyectos de Investigación?

- Who and what bodies oversee the related research budgets - Ministerio de Economía y Competitividad - MINECO?

- Understand what tools are available to you - briefings, hearings, report language, lobbying, press, legislation?

- Create a simple goal ‘low hanging fruit’

- Galvanize self-advocates and families with strong stories in voting districts or with personal relationships to reach simple and more aggressive, long-term goals

Of all the forms of inequality, injustice in health care is the most shocking and inhumane.

~ Martin Luther King Jr.
What Self-Advocates Should Consider

- **What is a good fit for you?**
  - Petition signing?
  - Writing to legislators?
  - Meeting with legislators?
  - Meet with government bodies providing funds for Down syndrome research?
  - Organizing small or large groups to do any and all of the above?
  - Follow and like relevant social media, send to your network AND post your own advocacy social media?

- **Can you do this?**
  - Becoming an advocate takes time and effort.
  - How much time do you have?
  - Can you do something once a week? Once a month? Once every three months? Once a year? ALL EFFORTS ARE IMPORTANT!
  - Commit to and stick to a plan...

- **Join/appropriate a Down syndrome organization**
  - Amplify your voice
THANK YOU from our GLOBAL ADVOCACY CHAMPIONS!

“Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has.”

Margaret Mead