



GLOBAL
DOWN SYNDROME FOUNDATION®

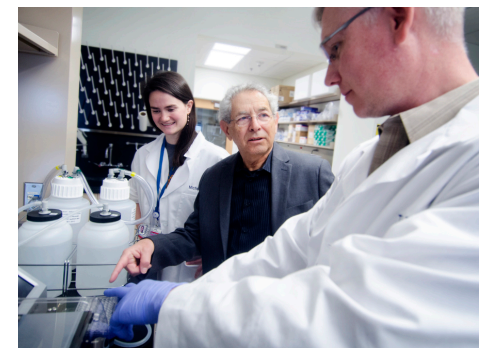
Advocates Fueling Our Progress in Research and Medical Care

Thursday, July 12, 2018

Michelle Sie Whitten, President & CEO Global Down Syndrome Foundation

Today's Presentation - We hope to answer some important questions...

- ❖ Who are our “Advocates”?
- ❖ What is research?
- ❖ How are research and medical care related and why are they so important?
- ❖ What have we accomplished so far? What is still to be done? How can you help advocate?
- ❖ Other Q&A
- ❖ THANK YOU!



Who are Global's advocates?

❖ What do we mean by “Advocacy?”

- An activity by an individual or group which aims to influence decisions within political, economic and social systems and institutions
- Helping to get legislation and funds that improve the lives of people with Down syndrome - RESEARCH and MEDICAL CARE Advocacy

❖ What do we mean by “Self-Advocates”

- Individuals with Down syndrome who are engaged in our Advocacy
- Everyone who helps us with our Advocacy! Families, Professionals, Volunteers, Donors, Lobbyists

Who are Global's Advocates?

Over 3,000 people including 100+ self-advocates who help us write letters, sign petitions, visit legislators and sometimes travel to DC with us!



What is Research?

❖ Basic Research

- Generates new ideas, principles and theories, which may stimulate new ways of thinking that has the potential to dramatically improve how medical professionals deal with a diagnosis or disease in the future.

❖ Translational Research

- Aims to “translate” Basic Research into medical practice - from laboratory experiments to clinical trials to point of care patient applications - in order to produce new drugs, devices and treatment options for patients.

❖ Clinical Research

- A branch of healthcare science that determines the safety and effectiveness of medications, devices, diagnostic products and treatment regimes intended for human use. As opposed to “clinical practice” using established treatments, evidence is collected to establish a treatment.
- Clinical Trials - Experiments or observations done in clinical research. Studies of people designed to answer specific questions about biomedical or behavioral interventions, including new treatments (e.g. novel vaccines, drugs, dietary choices/supplements and medical devices) and known interventions that warrant further study and comparison. Clinical trials operate data on safety and efficacy.

Wikipedia Search!

What is Research?

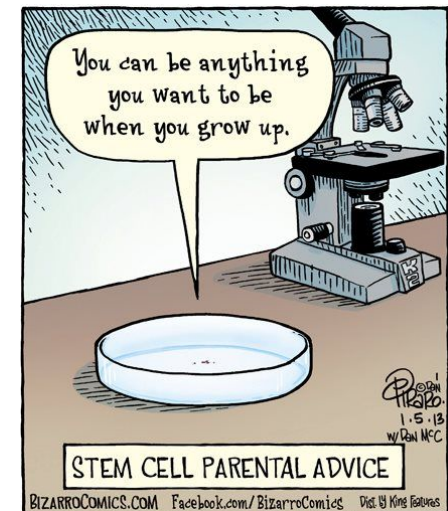
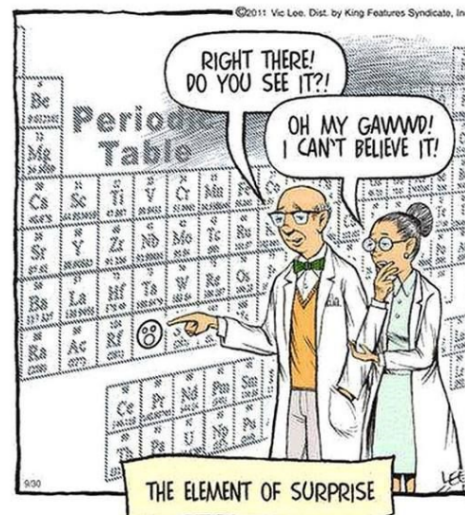
❖ Epidemiology

- A branch of medical science that deals with the incidence, distribution, and control of disease in a population.
- Natural History Research - The natural history of disease is the course a disease takes from its onset until its eventual resolution through complete recovery or death...It is a major element of descriptive epidemiology.

❖ Review of the Literature

- A scholarly paper...they are secondary sources, and do not report new or original experimental work. Most often found in academic journals. Sometimes they are included to provide context for a peer-reviewed journal article presenting new research.

Wikipedia Search!



Why are research and medical care so important?

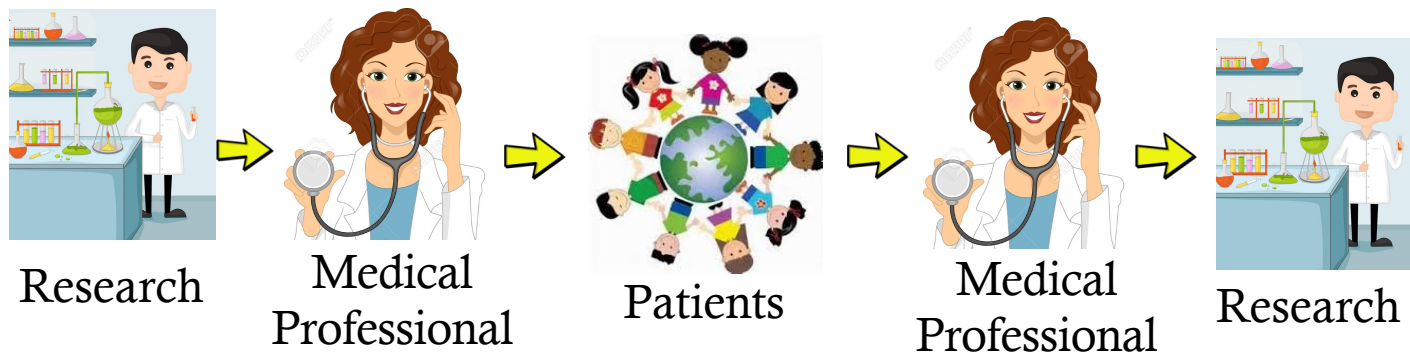
❖ Extends the lifespan

- In the 1980s lifespan was 28 years...today it is approximately 60
- Reasons - De-institutionalization + advances in pediatric heart surgeries

❖ Improves the quality of life throughout life

- ❖ 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)
- ❖ No current guidelines for adults with Down syndrome (Drs. William I. Cohen and David S. Smith)

❖ People with Down Syndrome have a Different Disease Spectrum!



Down Syndrome Research Overview

- ❖ **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).

The Critical Need

A Population Explosion & A Eugenics Framework

- ❖ There is a population explosion of people with Down syndrome in the U.S. that requires dramatically *more* funding not less
 - **Population** - is somewhere between 250,000 to 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...

Global - Proud of Our Accomplishments

RESEARCH

❖ Established the first research and medical care institute

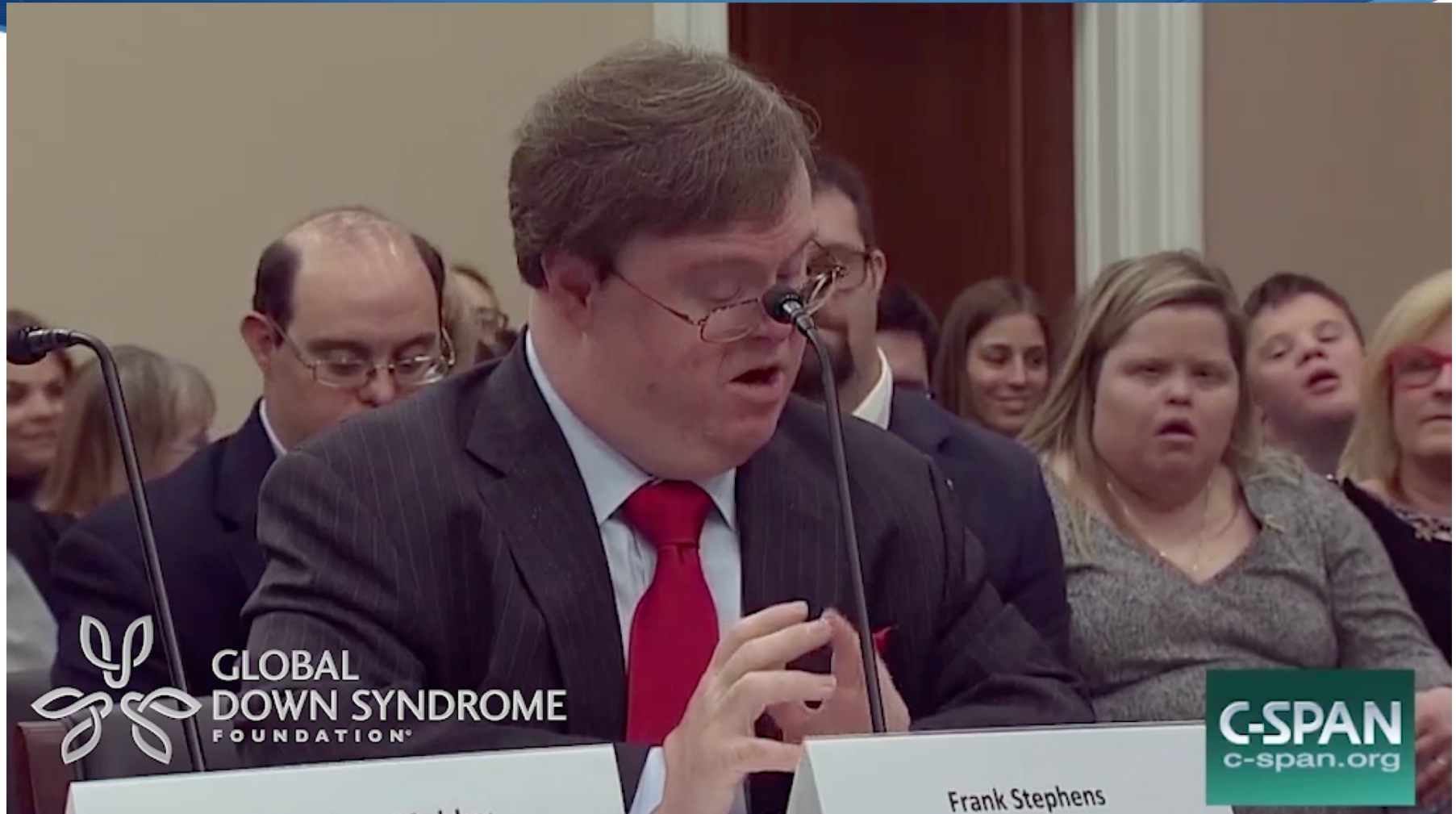
- Solely dedicated to improving the lives of people with Down syndrome - the Crnic Institute for Down Syndrome

❖ Lobbying and Advocacy in Washington DC

- Since 2005 and formally since 2009 with REPORT LANGUAGE resulting in:
- (Dec 2010) NIH and Global co-hosting the first Down Syndrome Conference in NIH history that led to the establishment of the DS-Connect patient registry
- The first real increase for Down syndrome research in nearly two decades - FY18 \$58M (up from \$35M), FY19 \$98M
- The establishment of a Trans-NIH Down Syndrome Research Consortium



Frank Stephens testifies Before Congress ... resulting in a historic 66% increase of NIH's Down syndrome research budget from \$38mil in FY2017 • to \$58 mil in FY2018 • to \$98mil in FY2019



Global - Proud of Our Accomplishments

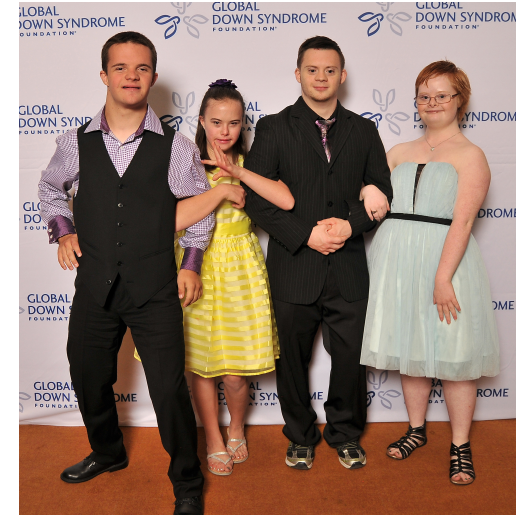
Medical Care

- ❖ **Established a Down syndrome pediatric medical care center**
 - ❖ One of the best/largest in the world - Sie Center for Down Syndrome
 - 1,500 unique patients from 28 states and 10 countries; 14 FTE, two first-in-kind programs - full-time education specialist and mental wellness clinic (8 clinics total)
- ❖ **Publish the Prenatal Testing Pamphlet About DS with NDSC**
- ❖ **About to establish an Adult Down Syndrome Medical Center**
 - Started with a pilot last month 1 day a week! Just a handful in the US...
- ❖ **Will help publish Medical Care Guidelines for Adults with Down Syndrome in 2019!**



How You Can Help Become a Global Advocacy Star!

- ❖ Sign Up!
- ❖ Choose Research and/or Medical Care Topics that are important to you
- ❖ Important Work That **MUST** get done!
 1. Advocate to ensure that NIH continues to increase funding for Down syndrome research EVERY year! Petition, thank yous, visits
 2. Advocate and/or help fund Medical Care Guidelines for Adults with Down Syndrome.
 3. Participate in research
 4. Sign up for DS-Connects
 5. Help us to establish Medical Centers of Excellence for Down syndrome funded



YOU Can Make a HUGE Difference!

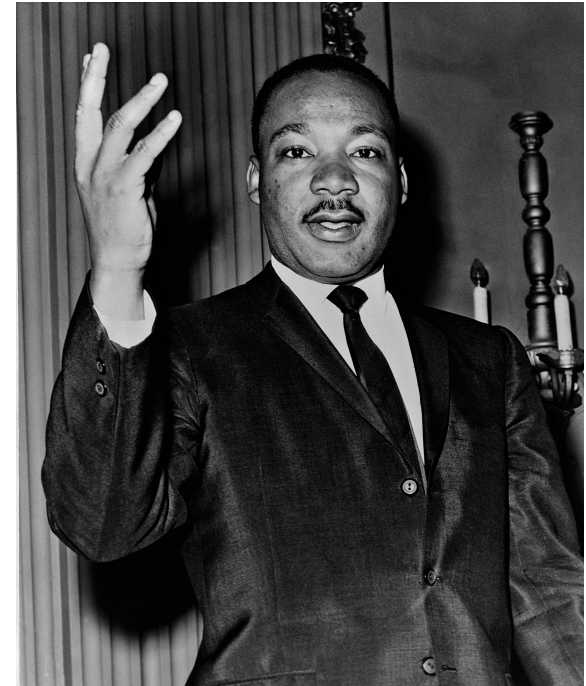


Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has.

~ Margaret Mead

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

~ Martin Luther King Jr.



Thank You from the GLOBAL ADVOCACY STARS

