Rare Disease State Advisory Council

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Establish a rare disease advisory council in your state

Tips from North Carolina’s journey
N.C. Council on Rare Diseases

Overview

- Signed into law by Governor Pat McCrory on August 5, 2015
- Passed by unanimous vote of both houses of North Carolina’s General Assembly
- Council is accountable to the General Assembly
- Domiciled at the University of North Carolina at Chapel Hill
- First meeting held on January 26, 2016
N.C. Council on Rare Diseases

Overview

- Members represent key voices in rare disease community:
  - Director – Bruce A. Cairns, MD, FACS - UNC School of Medicine
  - Associate Director - Tara Britt, UNC School of Medicine
  - Rare disease patient
  - Patient advocacy organization representative
  - Initially, research and clinical faculty from UNC & Duke
  - Eventually, all N.C. universities receiving specific NIH grants

- NOTE: Council can request approval from General Assembly to add representatives in the future.
N.C. Council on Rare Diseases

**Mandate**

- Leverage North Carolina’s resources and strengthen public & private partnerships to meet needs of rare disease patients
  - Research universities
  - Biotech industry
  - Statewide public & private research facilities
  - Healthcare delivery professionals and infrastructure

- Contribute to North Carolina’s economic development opportunities by creating quality jobs
N.C. Council on Rare Diseases

Current Status

- First report due to General Assembly in June 2016
- Identifying short- and long-term goals
- Identifying success metrics
- Improving data collection
- Identifying statewide opportunities
- Establishing collaboration framework
- Identifying technology platforms relevant for rare disease treatments
Identify state leaders’ priorities = JOBS
  - Governor’s Plan on Charlotte Chamber’s website

Taylor’s Tale President Sharon King meets with N.C. Representative Becky Carney on January 2, 2015, to seek advice for state house event in Raleigh

Rep. Carney advises:
  - State has specific guidelines = Legislators Breakfast
  - 20 minute presentation
  - Raise awareness about rare diseases against backdrop of state’s economic development priority
  - Legislators want to hear from N.C. constituents
  - Complete required paperwork to show NOT a lobbyist
Taylor’s Tale hosts Rare Disease Legislators Breakfast on March 25, 2015

- Approximately 50 legislators, rare disease advocates and biotech representatives attend
- Held in basement cafeteria of N.C. State House
- Previously postponed due to winter storm

Speakers:

- Sharon King, president of Taylor’s Tale
- Jon Stonehouse, CEO of BioCryst
- Jude Samulski, PhD, Director of Gene Therapy Center-UNC School of Medicine
Sharon King and Rep. Becky Carney welcome guests to Legislators Breakfast.
Dr. Jude Samulski reviews scientific progress.
Jon Stonehouse shares industry viewpoints.
Sharon King advocates for rare disease patients and their families.
North Carolina Rare Diseases Legislators Breakfast

Key Messages:
- Rare diseases statistics = public health imperative
- Emotional & financial impact on one family
- Economic impact to society & taxpayers
- Fund treatments = healthcare cost containment
North Carolina Rare Diseases Legislators Breakfast

Key Messages:
- Science is bearing fruit
- Business model is feasible
- N.C. public & private resources must collaborate to create economic prosperity
- Real people deserve real treatments
- Improve patient access to care
- Progress for one benefits ALL rare diseases

It’s a WIN, WIN, WIN!
N.C. Council on Rare Diseases Journey

- Breakfast generates strong bipartisan support
- Rep. Carney and King draft legislation with expert
- Rep. Carney recruits bill’s co-sponsors
  - 2 Democrats & 2 Republicans
- Governor’s Office and Secretary of Health and Human Services’ support confirmed
- King meets with approximately 50 legislators
  - Impromptu meetings in legislators’ offices
  - 3-minute speech to make key points
  - Target legislators’ priorities
- King meets with U.S. Senator Burr in Washington D.C.
N.C. Council on Rare Diseases Becomes A Reality

- April 27, 2015 - Bill passes House of Representatives *unanimously*
- July 1, 2015 - Bill passes Senate *unanimously*
- July 13, 2015 – Conference Committee approves revised bill
- July 28, 2015 – Revised bill passes House *unanimously*
- August 5, 2015 – Governor McCrory signs bill into law
N.C. Council on Rare Diseases Becomes A Reality

- Representative Marilyn Avila (center) introduced the bill for its final vote. She is pictured with Sharon King (left) and Tara Britt (right).
N.C. Council on Rare Diseases Becomes A Reality
N.C. Council Extends Outreach

Enlists expertise & engages rare disease advocates:

- Rare Diseases Executive Advisory
  - Subject-area experts recruited to advise Council with recommendations & assist with deliverables

- N.C. Rare Disease Coalition
  - Assist Council in identifying rare disease community issues
  - Serve as conduit for communication & advocacy
  - Ensure rare disease is a public health priority

(Executive Advisory & Coalition NOT created by legislation)
Tips for Success

Lessons Learned

- **TIP #1**
  - Each state is unique

- **TIP #2**
  - Research state’s priorities

- **TIP #3**
  - Understand state’s legislative process

- **TIP #4**
  - Abide by state’s rules and guidelines
Tips for Success

Lessons Learned

- **TIP #5**
  - Create a state-based rare disease advocacy coalition

- **TIP #6**
  - Build and cultivate relationships and partnerships

- **TIP #7**
  - Recruit champions in the legislature and administration

- **TIP #8**
  - Work with state experts to draft legislation for state-wide appeal
    - customize national models & resources
Tips for Success

Lessons Learned

- **TIP #9**
  - Aim for revenue-neutral legislation

- **TIP #10**
  - Engage state economic development offices/organizations

- **TIP #11**
  - Engage research universities

- **TIP #12**
  - Engage private sector (biotechs, etc.)
Tips for Success

Lessons Learned

TIP #13
- Effective messaging is essential
  - Do homework - know the facts
  - Focus messaging on state’s priorities
  - Focus messaging on individual legislator’s priorities
  - Focus messaging on benefits to citizens
  - Be succinct!
  - Be positive

TIP #14
- Set an expectation for collaboration
  - A win for one is a win for all

TIP #15
- Always remember: It’s about the patients!
Thank you.