



Comprehensive Care for Neurofibromatosis:

Can we make the transition?



Continued Care into Adulthood

- Genetic diseases and their complications are not limited to childhood.
 - Not just NF – also applies to CF, sickle cell, metabolic disorders, etc.
- Primary care physicians are often not trained to recognize and treat the complications.
 - Medical home concept for chronic diseases.
 - Requires continued dialogue.
- Loss of specialized care can clearly result in increased morbidity and mortality.
- Frustrating for both health care providers and for patients and families.



What are the barriers?

- Physical:
 - Rules and regulations at the clinics and hospitals.
 - Chronological age limitations.
 - Need for appropriate subspecialists.
 - Limited centers – long distances for patients.
- Providers limited and limitations:
 - Limitations of training.
 - Limited number of people.
 - Defining the scope of specialty care versus medical home.



Barriers continued:

- Inertia:

- Parents and patient comfort level with pediatric providers.
 - Survey of 122 transitional programs found family resistance was not commonly reported. Scal et al., 1999
- Physicians comfort with patients and their manifestations.
 - In a US population based study of parents of 5533 patients aged 13-17 with special health care needs, only 50% had talked about the changing needs in adulthood, 29% had a plan to address the needs and 21% had discussed shifting care to an adult provider. (Kennedy et al., 2008)

- Funding limitations:

- Funding resources exist for pediatrics.
- Limitations even for those insured.
 - Only one provider billing.
 - Limitations on testing.



Does Transitioning Care Really Matter?

- Hemophilia model:
 - 1975 Congress established with states a funding mechanism for a network of hemophilia treatment centers (HTCs). Initially established 22 funded by congress and 21 by states.
 - Expanded by funding from the CDC during the HIV outbreak in the 1980s.
 - Now with > 130 treatment centers throughout the US.



HTCs

- Established innovative treatment guidelines.
 - First to establish home infusion of factor.
 - Universal data collection – able to monitor outcomes and complications.
- Clearly demonstrated a decrease in hospital admissions, emergency room visits and mortality.
 - Baker et al., 2005; Evatt, 2006.
- Continues to push new treatment approaches and can track outcomes.
- Demonstrated the great benefit for treatment and follow-up throughout patients lives.



How to transition:

- Goal is to establish multidisciplinary teams.
 - Disease specific clinical centers.
 - Brings several subspecialists together in one setting.
 - Coordination by primary care physicians.
 - Requires active PCPs.
 - Requires a fair amount of time on the PCPs part.



How can it be done?

- External funding.
 - Hemophilia experience demonstrates external funding can help drive setup.
 - Grants for national, local and private entities.
- Centers of excellence approach at major medical centers.
 - Drive to provide cutting edge care expertise.
 - Often clinical trials oriented approach.



Our approach:

- Center of Excellence branding:
 - Benefits:
 - Strong basic science on neurofibromatosis at UTSW.
 - Utilize that research to streamline translational trials – the bench to bedside dream.
 - Requires dedicated and vocal champion.
 - Semi-multidisciplinary, team approach.
 - Limitations:
 - Funding! - still not open to all patients.
 - Is specialized care.
 - Is provider driven.
 - Different systems, nurses, etc.



Questions?