RDAC Transitional Care Sub-Committee: 10/23/2025

I. Overview

Pursuant to the Massachusetts Rare Disease Advisory Council (RDAC) charter, we present this summary of key aspects of transitional care for adolescents and young adults (AYAs) affected by rare disorders. Transitional care (TC) is not simply the transfer of care to a new healthcare provider. Rather, TC describes the purposeful, planned process for moving an AYA from pediatric to adult-oriented care (with or without a change to a new healthcare provider). The document provides the legislature with a summary of the effects of TC on health outcomes, wellbeing, healthcare utilization, and cost.

Transition to adult-oriented care is a salient issue for all AYAs in the Commonwealth. Approximately 1 in 4 young adults live with a chronic health condition and all will have to transition to adult care at age 18 yrs. For those with rare diseases (i.e., conditions affecting fewer than 200,000 individuals) transition challenges are magnified by medical complexity, limited provider expertise, and fragmented care systems. The lack of purposeful, planned TC creates gaps and cracks in care resulting in poor outcomes for health and wellbeing. Moreover, lack of adequate TC can increase direct healthcare costs (e.g., emergency department visits, hospitalizations, complications requiring intensive treatment), lost productivity (e.g., absenteeism), and long term system burden (e.g., dependence on public assistance programs).

There are numerous TC stakeholders including patients and families, healthcare providers (both pediatric and adult-oriented), health systems, and payers. Existing literature supports the benefits of TC in addressing the “triple aim” of healthcare: improving health, enhancing experiences with healthcare, and reducing costs. Evidence-based guidelines and frameworks are available providing structured approaches for effective implementation.

II. Evidence Base

Research has long demonstrated that lack of structured TC leads to adverse outcomes including:

- Gaps in care (i.e., decreased clinical attendance and discontinuity of care)
- Disrupted disease management (i.e., medications and therapies)
- Complications and poor symptom control
- Increased utilization of emergency care and hospitalizations
- Impact on mental health (i.e., anxiety, depression, diminished quality of life)
- Increased direct healthcare costs, lost productivity, and increased health system burden resulting from high utilization

In contrast, purposeful, planned TC results in:

- Continuity of care and fewer missed appointments
- Enhanced self-management skills
- Reduced emergency utilization and fewer hospitalizations
- Improved long-term health outcomes
- Greater patient satisfaction

III. Challenges for TC in rare diseases

Studies have examined TC for sickle cell disease, cystic fibrosis, congenital heart defects, spina bifida, as well as rare rheumatologic and endocrine disorders. Challenges include:

- Difficulty accessing expert care. Geography is a significant barrier for patients and families.
- Lack of adult "receiving" provider. Most rare diseases are identified in childhood, so many adult providers are reluctant to accept AYAs with rare diseases.
- Differing orientation and models of care. Pediatrics is family-centered while adult care emphasizes autonomy.
- Financial complexity. Families must navigate complex insurance coverage, disability benefits, and long-term financial planning during transition.
- Lack of coordination. Vital information, care plans, and specialty care can be lost without structured TC.
- Lack of adult guidelines. Many rare disorders lack adult-specific treatment guidelines leading to uncertainty regarding dosing, monitoring, and managing complications.
- Fragmented care. Patients must often navigate and independently coordinate their own care across multiple, siloed specialties
- Timing. Age cutoffs (typically 18 yrs., 21, 26…) for pediatric care may force transitions regardless of developmental readiness or medical stability (e.g., intellectual and/or behavioral issues). Parents/caregivers may be abruptly excluded from medical decision-making when the child becomes a legal adult.
- Lack of support. The transition can be a very stressful time for AYAs and there is generally a lack of staff to support families and help them navigate the often-difficult passage to adult-oriented care. Case management (transportation, housing, finances, etc.)
- Medical independence/autonomy: Medical proxy does not equal executor! (medical-legal complications)

Improving transitional care represents a unique opportunity to enhance healthcare quality, reduce unnecessary costs, and improve long-term outcomes for AYAs with rare diseases. By addressing systemic barriers through coordinated policy action, the legislature can help ensure these vulnerable patients don't fall through the cracks during this critical life transition.

IV. Ideating Possible solutions (draft ideas for discussion)

Based on our discussion, I envision that we would advocate for structured transition programs that would ideally offer:

- Dedicated transition coordinator / patient navigator (e.g., nurse, APRN, social worker)
- Programs co-designed with key stakeholders
- Purposeful, planned and flexible (e.g., age) process
- Leverage digital technologies to foster/support communication
- Person-centered / family-centered approach
- Assessment of transition readiness & self-management
- A regular review of transition needs

- Co-location of multidisciplinary teams

Possible recommendations?

1. Endorse standards

- Establish transitional care standards (e.g., American Academy of Pediatrics / Got Transition "Six Core Elements of Transition" framework)
- Require healthcare organization to establish formal TC policies
- Create quality metrics specific to TC to codify “success”
- Case management & PCP roles
- Legal consultation & planning (lifespan issues / probate)

2. Enhance reimbursement mechanisms

- Incentivize joint pediatric-adult provider consultations
- Create TC-specific billing codes or bundle payments for successful transition
- Fund TC care coordination (navigator) positions

3. Address structural barriers

- Virtual/telehealth to extend access to rare disease expertise
- Protect/extend insurance and specialty coverage for AYAs during transition (18-26 yrs.)
- Create TC clinics with dual pediatric/adult staffing and multidisciplinary teams
- Coverage for cross-border or interstate visits/consultations

4. Build and strengthen workforce capacity:

- Support adult-oriented primary care providers in managing patients with rare diseases
- Fund/support continuing education for healthcare providers on rare disease management
- Fund fellowship training in TC, and navigator training for nurses/advanced practice providers
- Include TC content in medical and nursing curricula and board exams

5. Develop information technology infrastructure

- Prioritize interoperable electronic health record systems
- Create and endorse standardized TC summary templates
- Support patient access and control of electronic health records

6. Fund TC research and networks

- Allocate funding for TC outcomes research
- Create TC quality improvement collaboratives/networks (Project ECHO)
- Develop metrics for TC quality assessment (HEDIS)

Data sources

This summary is informed by a review of current TC literature as well as testimony and presentations given to the RDAC and ensuing discussion with RDAC members.

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