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Texas Rare Alliance

TX Rare 2021

Legislative Priorities

TX Rare State of Access

The top priority of TX Rare for the 87th Texas Legislative Session is to make Texas a **State of Access** for the rare disease community



What Is Access?



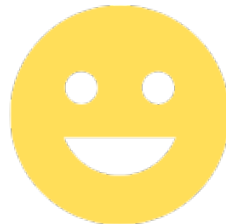
- Diagnosis
- Medical Services
- Therapeutics
- Insurance Coverage
- Funding



Copay Accumulator Ban

- Accumulator programs allow insurers to collect Patient Assistance Program (PAP) funds without crediting copays and deductibles as intended
- Patients exhaust the PAP funding and are still liable for satisfying the copay and deductible before accessing the next treatment
- Accumulator programs essentially allow insurers to collect copay and deductibles twice
- Negatively impacts patient access
- Decreases patient adherence

Mortgage Holder



Insurer

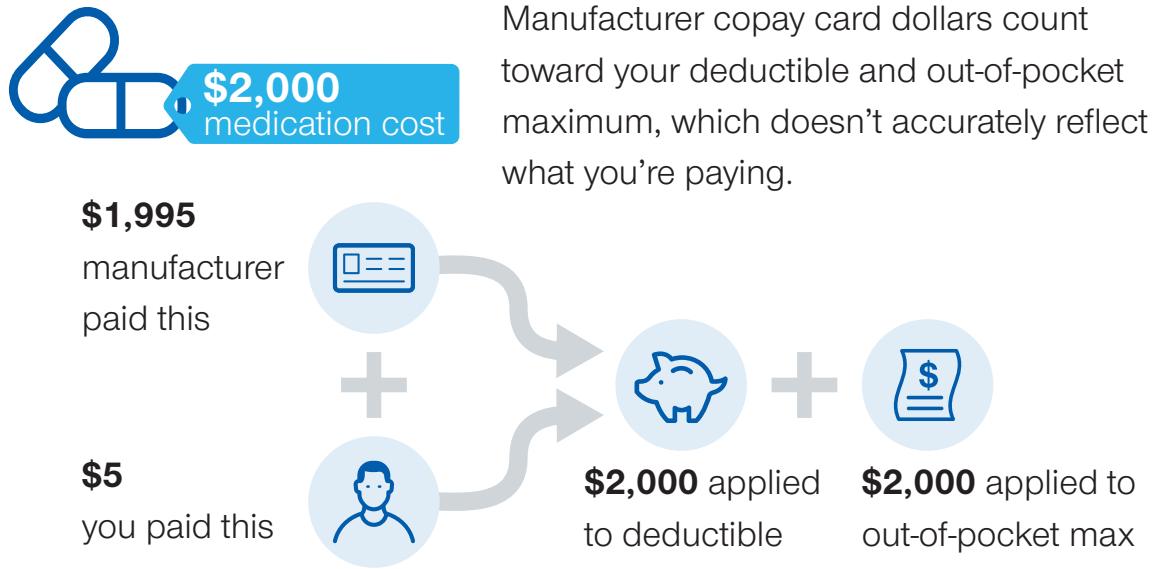


PAP



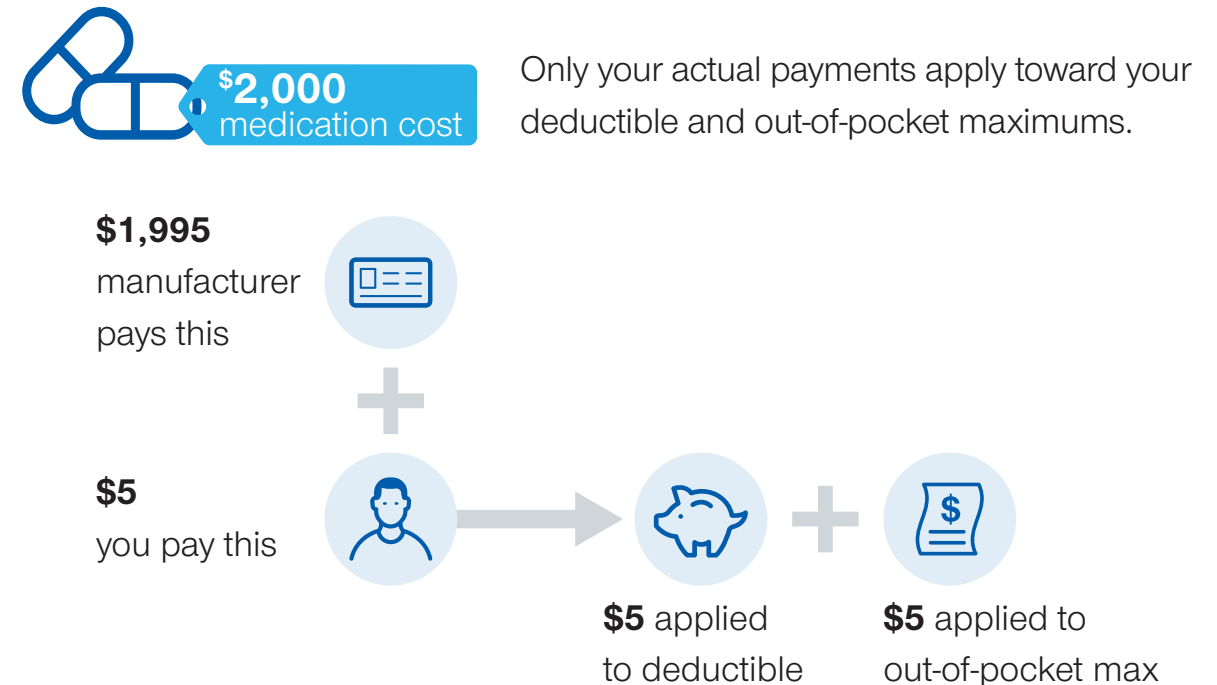
How Accumulator Programs Work

How it worked before



- Patient paid \$5, manufacturer paid \$1,995, & \$2,000 out of pocket max is satisfied
- Insurer collected \$2,000

How it works with the new program



- Patient paid \$5, manufacturer paid \$1,995, & \$5 out of pocket max is satisfied
- Patient must pay \$5 copay and \$1,990 toward deductible for out-of-pocket max for refill
- Insurer stands to collect \$3,995

Potential Impacts of Accumulator Programs

- ↑ Medicaid waiver applications
- ↑ Medicaid spend downs
- ↑ Acute health crises
- ↑ Reliance on food banks – 1/3 of participating households have had to choose between paying for food and paying for medicine or medical care
- ↑ Government healthcare spending on medications
- ↑ Requests to nonprofit assistance funds
- ↑ Mortgage foreclosures
- ↑ Missed work by patients and caregivers
- ↑ Medical service spending

It's a Sophie's Choice Scenario

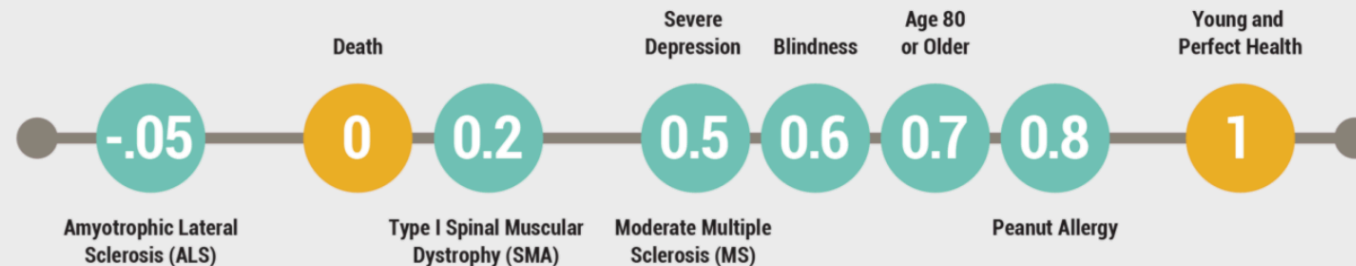


QALY Education

- QALY metrics devalue of lives of medically fragile, disabled, and elderly patients
- The diminished value of patient lives is used to justify decreased reimbursement of treatments
- QALY metrics are ableist and discriminatory tactic arguably violating the ADA
- Educate Texas lawmakers on the dangers QALYs pose to patients

QALY Scores

Quality-Adjusted Life Years (QALYs) are a number which (theoretically) represents the degree to which a drug or treatment extends life and improves quality of life. The QALY assigns a value to a human's life on a scale between 0, dead; and 1, perfect health. Are you worth treating?





I'm More Than

0.2

Hunter is 9 and loves spending time with his family. He's the youngest of 5 children. Hunter enjoys watching SpaceX rockets with his dad, learning about robots, and following his friend and hero, Winter the tailless dolphin.

Hunter has Spinal Muscular Atrophy Type 1, the leading genetic cause of death of infants.

Hunter is much more than his QALY of 0.2. QALYs are dangerous for vulnerable TX patients like Hunter.

Hunter Davis, Austin

QALY Ban

- Massachusetts & New York currently use QALY metrics to value treatments covered in Medicaid programs
- Oklahoma enacted legislation prohibiting the use of QALY metrics in its Medicaid program
- Secure a QALY Ban for Texas Medicaid Programs
- Value therapeutics on outcomes and other measures, not with metrics that devalue the lives of vulnerable Texas patients

Newborn Screening Funding



- Advocate to secure funding to implement SMA NBS in June - SMA was added to the RUSP in July of 2018
- Advocate to secure funding for implementation of Pompe and MPS I NBS in 2024 – building retrofit, FTEs, equipment, and reagents
 - Pompe was added to the RUSP in March of 2015
 - MPS I was added to the RUSP in February of 2016
- Advocate to secure funding for a study on NBS laboratory space and feasibility for screening future conditions with treatments in clinical trials – Includes Batten Disease & Rett Syndrome

Project Baby Dillo

- PBD will provide rWGS to low income NICU and PICU patients with unknown etiologies
- 300 conditions would qualify for the RUSP inclusion if they had a genetic test
- Minutes matter when NICU and PICU patients present with undiagnosed conditions
- PBD would improve health outcomes in critically ill newborns and children while decreasing healthcare spending and number of hospital days spent in the NICU and PICU for eligible patients



Project Baby Dillo



Nonmedical Switching

- Advocating to secure legislation that will allow Texas patients to stay on medications already working for them, as long as they remain on the same health plan
- Allows patients to predict their out-of-pocket costs of their medications from year to year when they're on the same health plan
- Allows the substitution of generics
- Allows the removal of medications deemed unsafe or ineffective by the FDA



Telemedicine



- Plays an increasingly important role as many clinical trials become decentralized
- Protects the rare disease community from acquired conditions like Covid-19
- Allows patients in rural areas to access clinicians with a substantially decreased burden
- Helps address health inequities by improving access for patients with low income, no transportation, and mobility challenges
- Requires broadband and smart devices – access barrier for some

TX Rare Virtual Rare Disease Week

- Rare Disease Day is Sunday February 28, 2021
- TX Rare Virtual Rare Disease Week – March 1st-5th
- Proclamations recognizing Rare Disease Day
- 4-6 virtual advocacy meetings a day with Texas Representatives, Senators, and staff
- A virtual meeting celebrating the Texas Rare Disease Community





TX Rare State of Access Program

- State of Access Podcast
- State of Access Webinars
- State of Access Newsletter

ARM Foundation Texas STRiDE RDEF Steering Committee

- Conduct landscape analysis on access to cell and gene therapies in Texas, beginning with Medicaid
- Develop Texas-specific advocacy documents and tools to help educate and improve access for cell and gene therapies

ARM Foundation Texas STRiDE RDEF Steering Committee Members

- **Nora Belcher**, Executive Director, Texas e-Health Alliance
- **Dennis Borel**, Executive Director, Coalition of Texans with Disabilities and DUR Board Member
- **Titilope Fasipe**, Assistant Professor, Department of Pediatrics, Hematology/Oncology, Texas Children's Hospital
- **Steven Gray**, Associate Professor, Department of Pediatrics, UT Southwestern
- **Julie Haeber**, Associate Director State Government Affairs, Ultragenyx
- **Tom Kowalski**, President & CEO, THBI
- **Whitney Lavender**, Director of Community Engagement, Texas Rare Alliance
- **Premal Lulla**, Assistant Professor of Medicine, Hematology-Oncology, Center for Cell and Gene Therapy, Baylor College of Medicine
- **Emily McGinnis**, Director of Policy & Patient Advocacy, Taysha Gene Therapies
- **Rob Osborne**, Vice President Specialty Pharma Trade Relations, Acreedo
- **Jess Rabourn**, Expanded Access Expert, Wide Trials
- **Cecilia Ruberto**, Alliance Development Manager –Texas, New Mexico, and Oklahoma
- **Kate Segal**, Director State Government Affairs, Sarepta
- **Brian Shank**, Director, State Government Affairs, Alexion Pharmaceuticals
- **R'Kes Starling**, CEO & Founder, Reveles Clinical Services
- **John Williamson**, Senior Patient Advocate, HAEA

Thank You!

Texas Rare Alliance is dedicated to improving access and health outcomes for nearly 3 million Texas rare disease patients through education and advocacy.

