Real Life™

Medical Affairs Policy Update

AAWG

October 11, 2022

CIRM
CALIFORNIA'S STEM CELL AGENCY

Cell and Gene Therapies - Background

- There are 24 approved cellular and gene products approved by the FDA. It is projected there will be 10-20 new cell and gene therapy approvals each year
- There are approximately 2,200 active clinical trials in cell and gene therapies in several therapeutic areas
- There are a several federal and state legislative activities that CIRM is following that could impact patient barriers to Cell and Gene Therapies, particularly the underserved patient populations
- The purpose of this presentation is to provide the first of several updates on selected legislation that may impact patient access and or affordability to Regenerative Medicines

We Continue to Address Barriers to Achieve Broad, Equitable Access to Regenerative Medicines

CULTURAL AND SOCIAL DETERMINANTS

- Lower enrollment for minorities [2]
- Socioeconomic status, unemployment, education [4,5]
- Population size/geography [6,7]
- Stigma of disease [8,9]

INFORMATIONAL

- Physician low referral rate [10]
- Medical mistrust and misinformation about regenerative medicine [11]

LOGISTICAL

- Lack reliable transportation [12,13]
- Language[14]
- Work or childcare requirements [11]

FINANCIAL

- Cost of regenerative medicines (gene or cell therapies) and insurance benefits may include high copays and lifetime benefit [15]

ABILITY-BASED

- Participation is limited for elderly,[16] adolescent and young adult,[17] and disabled patients[18,19]

Legislation that Could Impact Access and Affordability

TREATMENT

DISTRIBUTION

PRICING AND REIMBURSEMENT

MANUFACTURING

Selected Policy Examples

- California Cancer Care Equity Act (SB 987) Signed/Law – expands access to specialized cancer care centers (NCI) for Medi-Cal patients
- Inflation Reduction Act - Signed/Law – allows Medicare to negotiate “best price” and performance-based contracts (limited) and still won’t take effect until 2023 or 2024.
- Food and Drug Act of 2022 (HR 7667) - user fee, increase diversity in trials, improves supply chain
- Start-ups for Cures Act (HR 7504) – allows small companies to put more of their capital toward working to prepare for the health care challenges of tomorrow
- Infectious Disease Therapies Research & Innovation Act (HR 7515) - encourages earlier investment and stronger research in critical therapies and vaccines
- More Cures Act (HR 7505) – creates 14% bonus R&D tax credit for companies engaged in drug development research, particularly life-saving drugs
- CMS-1771-P – Inpatient payment and updating NTAP reimbursement for gene-edited technologies
- HR 6000 – Pandemic preparedness and genetic testing
- American Made Medicine Act (HR 7410) – Provides tax incentives to encourage domestic manufacturing of pharmaceutical ingredients

Areas for AAWG Consideration to Improve Access to CGT

- Performance-based agreements for private and public payers are generally well-accepted but not clearly defined
- Federal and State formulary reviews for CGT often take time ~180-360 days
- Concern that many patients will require therapy across state lines and Medicaid payments will be delayed
- Concern there will be a differential reimbursement for outpatient vs inpatient treatment further impacting patient co-pays
- Will new state accreditation for hospitals limit gene therapy administration?
- Will new state regulations limit distribution of cell and gene therapies thus impacting patient access?

Areas for AAWG Consideration to Improve Access to CGT

- How can we further assist cancer patients under California's Cancer Care Equity Act
- How does CMS regulations authorizing access and reimbursement for clinical trials provide access to cell and gene therapies
- How can the Alpha Clinics Network streamline access and facilitate reimbursement in this evolving policy landscape

Recommendations Moving Forward

- Develop a Dashboard of Policy and Legislation to present to AAWG on a monthly basis. Dashboard will include:
  - Legislative Bills and Initiatives
  - Policy Briefs
  - Professional Society Guidelines and Reports
  - Published Literature
  - State and Federal Guidance Documents
- Other AAWG Recommendations?