Trends in Cell and Gene Therapy Uptake and Utilization in the United States: A Real-World Analysis of the Komodo™ Healthcare Map Claims Data from 2015-2021

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RWD93

ISPOR Annual 2022

Summary

- Real-world use across cell and gene therapies (CGT) is not well quantified in currently published literature
- This study aimed to evaluate CGT uptake and quantify utilization patterns in the US using claims data in the Komodo Healthcare Map™
- CGT utilization has steadily grown in the US, providing innovative, and in some cases curative, treatments across many conditions, many of which offered no prior treatment options to patients

Introduction & Objectives

- Cell therapy is the transfer of live, intact cells into patients to treat/cure a disease[1]
- Gene therapy is the use of genetic material in the treatment/prevention of a disease[1]
- CGTs have fundamentally different properties than medications and surgical procedures, and also have the potential to cure patients[2]
- Cell and gene therapies (CGT) represent tremendous innovation in the life sciences landscape and have captured national attention. However, the real-world use across this therapeutic class is not well quantified
- This study aimed to evaluate CGT uptake and quantify utilization patterns in the US between 2015 and 2021 at the time of analysis

Methods

- This study analyzed patient-level US claims data in the Komodo Healthcare Map™[3], a dataset including longitudinal medical and prescription claims encompassing adjudicated claims of >320 million unique enrollees from the US commercial, Medicare, and Medicaid insured populations
- Patients were eligible for inclusion if they had ≥1 claim including the identified National Drug Codes or HCPCS Level II codes following drug approval dates
- Of the 22 FDA-approved CGTs, seven therapies were analyzed based on data availability contingent on approval dates, launch timing, and/or visibility of codes in claims data (see data notes for additional details). The analyzed CGTs, outlined in Table 1, included: IMLYGIC®, KYMRIAH®, LUXTURNA®, MACI®, PROVENGE®, YESCARTA®, and ZOLGENSMA®
- Data analyzed were representative of January 1, 2015, to December 14, 2021
  - Descriptive statistics for patient-level analyses were reported as means, standard deviations (SD), frequencies, and percentages, as appropriate
  - Due to the claims processing time lag, capture of the last six months of 2021 is incomplete
- Analysis on HCRU (Table 3) was conducted on a subset of patients with a 12-month lookback period prior to CGT initiation; patients are required to be continuously enrolled in the 12-month observation window to ensure complete capture of all healthcare checkpoints

Results

Table 1 | List of FDA-approved CGTs Included in Analysis[4]

Brand & Generic	Indicated For	Approval Date
ZOLGENSMA®[5] (onasemnogene abeparvovec-xioi)	Pediatric patients less than 2 years of age with spinal muscular atrophy (Type I) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.	May 24, 2019 (initial)
LUXTURNA®[6] (voretigene neparvovec-rzyl)	Patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Patients must have viable retinal cells as determined by the treating physician(s).	December 19, 2017
YESCARTA®[7] (axicabtagene ciloleucel)	Adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma. Not indicated for the treatment of patients with primary central nervous system lymphoma.	October 18, 2017 (initial)
KYMRIAH®[8] (tisagenlecleucel)	1) Patients ages 3-25 years with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse. 2) Adult patients with relapsed or refractory (r/r) large B-cell lymphoma after two or more lines of systemic therapy including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma.	August 30, 2017
MACI®[9] (Autologous Cultured Chondrocytes on a Porcine Collagen Membrane)	Repair of single or multiple symptomatic, full-thickness cartilage defects of the knee with or without bone involvement in adults. MACI is an autologous cellularized scaffold product.	December 13, 2016 (initial)
IMYLGIC®[10] (talimogene laherparepvec)	Local treatment of unresectable cutaneous, subcutaneous, and nodal lesions in patients with melanoma recurrent after initial surgery.	October 27, 2015 (initial)
PROVENGE®[11] (sipuleucel-T)	Treatment of asymptomatic or minimally symptomatic metastatic castrate resistant (hormone refractory) prostate cancer.	April 29, 2010

Table 2 | Sample Characteristics

Patient Sample Size (n)	21,198
Age in Years (mean [SD, Range])	66.8 (16.4, 1-89)
Female (%)	24%*

** Female (%) is affected by sex-specific indications and is primarily driven by the large PROVENGE® sample size.*

Figure 1 | Top Physician Specialty Prescribing Each CGT by Number of Patients

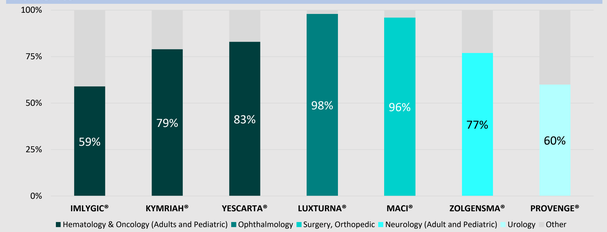
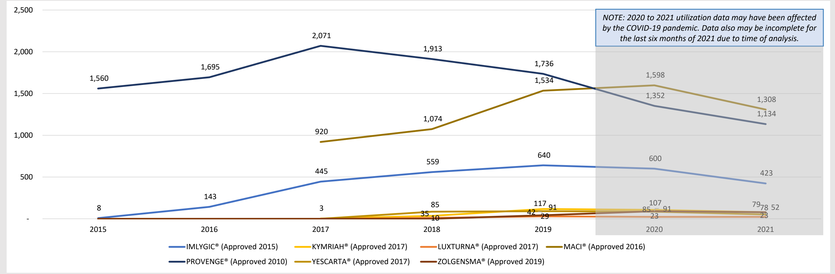
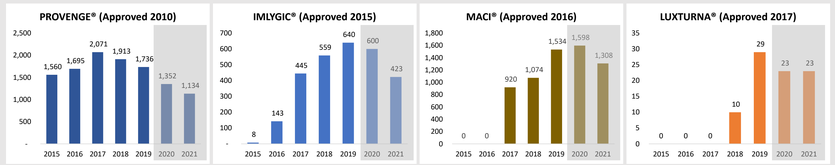


Figure 2 | Uptake Trends Over Time for CGTs (2015-2021)





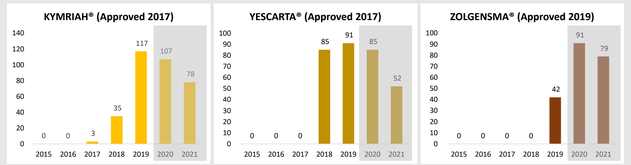


Figure 3 | Distribution of Patients Receiving CGT by Payer Type

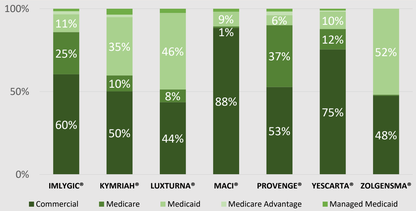


Table 3 | Healthcare Resource Utilization (HCRU) 12 Month Prior to CGT Initiation

Patient Sample Size (n)	5,510
Median Number of Inpatient Visits	2
Median Length of Stay (LOS) for Inpatient Visits (days/per Admission)	2
Median Number of Outpatient Visits	24
Median Number of Emergency Room Visits	2

Discussion & Conclusions

- Since 2015, CGT utilization has increased overall since more CGTs have been introduced, providing innovative treatments across many conditions, many of which had high unmet needs and underserved populations
- Authors hypothesize that declines in 2020 and 2021 may be due to COVID-19's impact on CGT uptake, incomplete data toward the end of 2021, patient warehousing in select cases, and potentially competitive pressures
- Patients now have potentially curative therapies for a variety of diseases, which thus far were life-threatening and/or severely limited a patient's ability to function, impacted their Quality of Life (QoL), and led to high healthcare utilization
- Future research should include analyses of the clinical outcomes of patients on CGTs, impact on their QoL, HCRU, cost of care, competitive landscape of CGTs within indications, and comparison to non-CGT treatments

Limitations

- This analysis had small sample sizes given the rare disease indications included and that many CGTs were approved recently
- Data may be incomplete for the last six months of 2021 as a result of the lag between data creation and availability due to claims processing
- The HCRU analysis grouped together patients from multiple therapeutic areas, preventing indication-specific comparisons
- This analysis is subject to selection bias in that it includes patients in the US with healthcare insurance and may not be representative of other populations

Data Notes

ABECMA® (idecabtagene vicleucel), ALLOCORD (HPC, Cord Blood), BREYANZI® (lisocabtagene maraleucel), CARVYKTI™ (ciltacabtagene autoleucel), CLEVECORD (HPC, Cord Blood), DUCORD (HPC, Cord Blood), GINTUIT (Allogeneic Cultured Keratinocytes and Fibroblasts in Bovine Collagen), HEMACORD (HPC, Cord Blood), HPC Cord Blood, HPC Cord Blood (MD Anderson Cord Blood Bank), HPC Cord Blood (LifeSouth), HPC Cord Blood (Bloodworks), LAVIV® (Azficel-T), RETHYMIC® (allogeneic processed thymus tissue–agdc), STRATAGRAFT® (allogeneic cultured keratinocytes and dermal fibroblasts in murine collagen- dsat), and TECARTUS® (brexucabtagene autoleucel) are also FDA-approved CGTs, but were not included in this analysis due to lack of visibility in the claims data. RETHYMIC® (allogeneic processed thymus tissue–agdc) was not yet widely available/marketed at the time of data analysis. Our analysis includes data for CGTs from year of approval and onward.

Data Statement