

What Makes a Drug “Special?”

Defining Specialty Medications



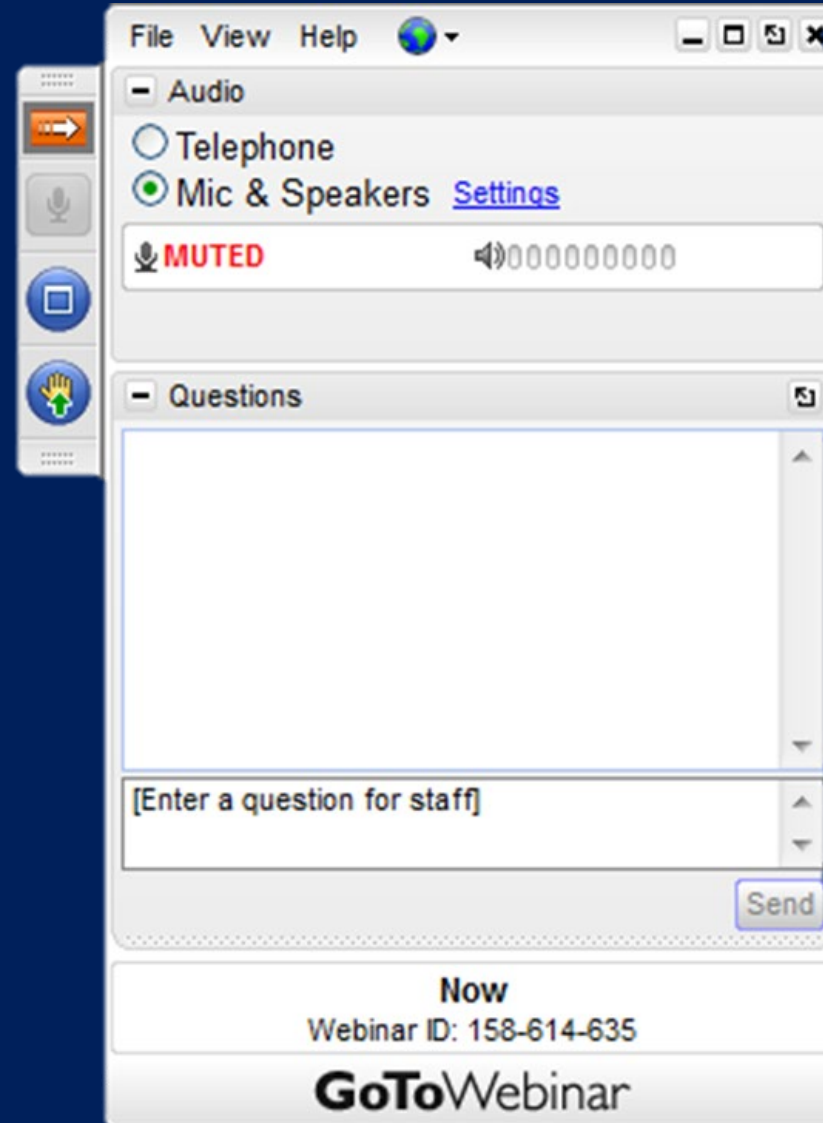
Steven Kheloussi, PharmD, MBA

Assistant Professor of Pharmacy Practice, Wilkes University
Senior Pharmacist, Clinical Pharmacy Strategies, Highmark, Inc.

Disclaimer

Organizations may not re-use material presented at this AMCP webinar for commercial purposes without the written consent of the presenter, the person or organization holding copyright to the material (if applicable), and AMCP. Commercial purposes include but are not limited to symposia, educational programs, and other forms of presentation, whether developed or offered by for-profit or not-for-profit entities, and that involve funding from for-profit firms or a registration fee that is other than nominal. In addition, organizations may not widely redistribute or re-use this webinar material without the written consent of the presenter, the person or organization holding copyright to the material (if applicable), and AMCP. This includes large quantity redistribution of the material or storage of the material on electronic systems for other than personal use.

How to Ask a Question



What Makes a Drug “Special?”

Defining Specialty Medications

Steven Kheloussi, PharmD, MBA

Assistant Professor of Pharmacy Practice, Wilkes University
Senior Pharmacist, Clinical Pharmacy Strategies, Highmark, Inc.

Learning Objectives

1. Determine the characteristics that classify drugs as specialty medications
2. Explain the roles of specialty pharmacies
3. Describe basic benefit design and channel management strategies for specialty medications
4. Discuss various aspects of biosimilars, including naming mechanisms and limitations to market uptake

Specialty Drugs

- No universal definition
- Drug-specific characteristics lead to specialty designation
- Why does it matter?
 - Coverage implications
 - Distribution implications

Characteristics of Specialty Drugs

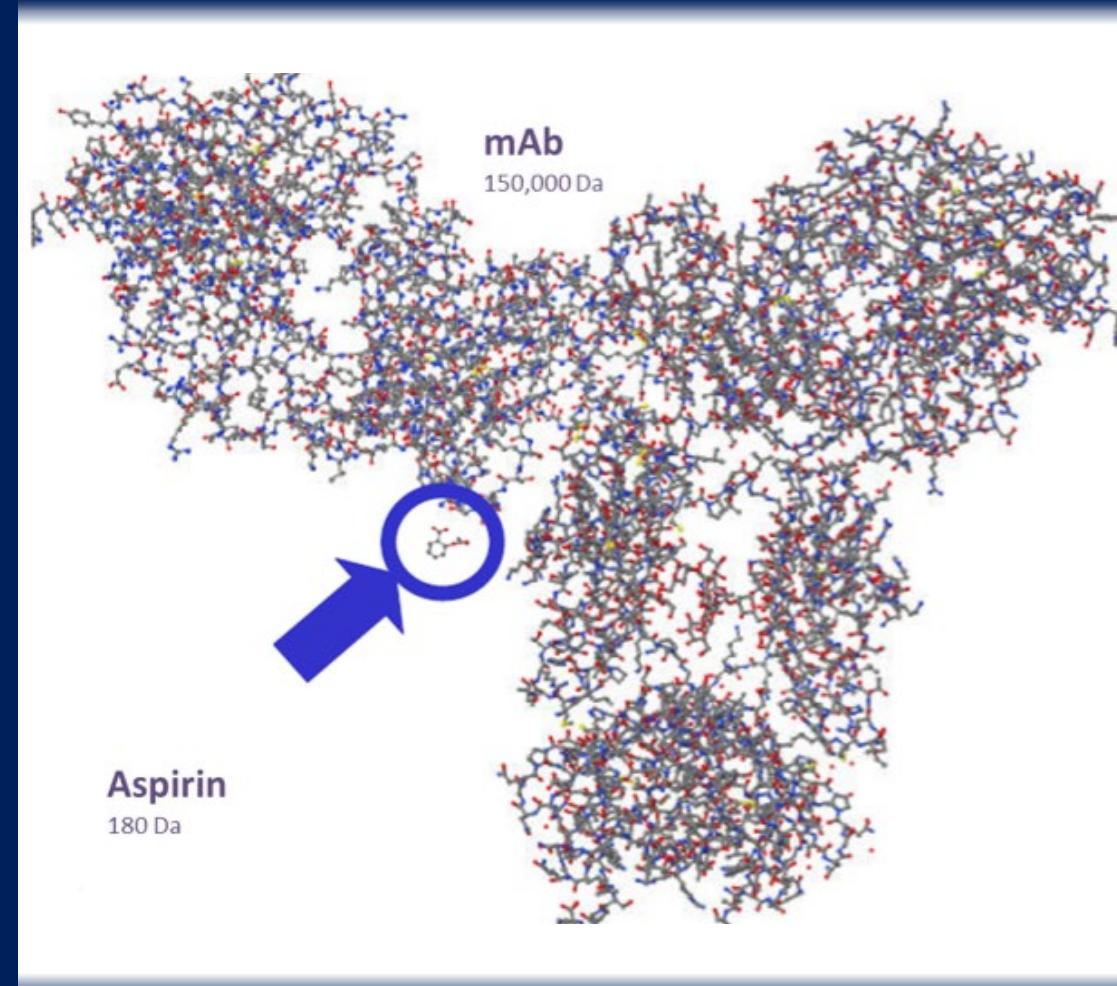
- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Biotechnology Manufacturing Process

- Complex, large, unstable molecules
- Often derived from living organisms



Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- **Special handling requirements**
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Special Storage/Handling Requirements

- Some products must stay refrigerated, frozen



Special Storage/Handling Requirements

- Some products must stay refrigerated, frozen



Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- **Customized dosing**
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Customized Dosing

Dosing

- Body surface area
- Blood levels
- Genetic factors

Total lifetime doses

- Doxorubicin max lifetime dose $< 550 \text{ mg/m}^2$

Customized Dosing

- Intralesional Injection

Injection Volume Based on Lesion Size	
Lesion size (longest dimension)	Injection volume
> 5 cm	Up to 4 mL
> 2.5 cm to 5 cm	Up to 2 mL
> 1.5 cm to 2.5 cm	Up to 1 mL
> 0.5 cm to 1.5 cm	Up to 0.5 mL
≤ 0.5 cm	Up to 0.1 mL

Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- **Complex delivery methods**
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Common Drug Delivery Methods

Oral

Inhalation

Topical

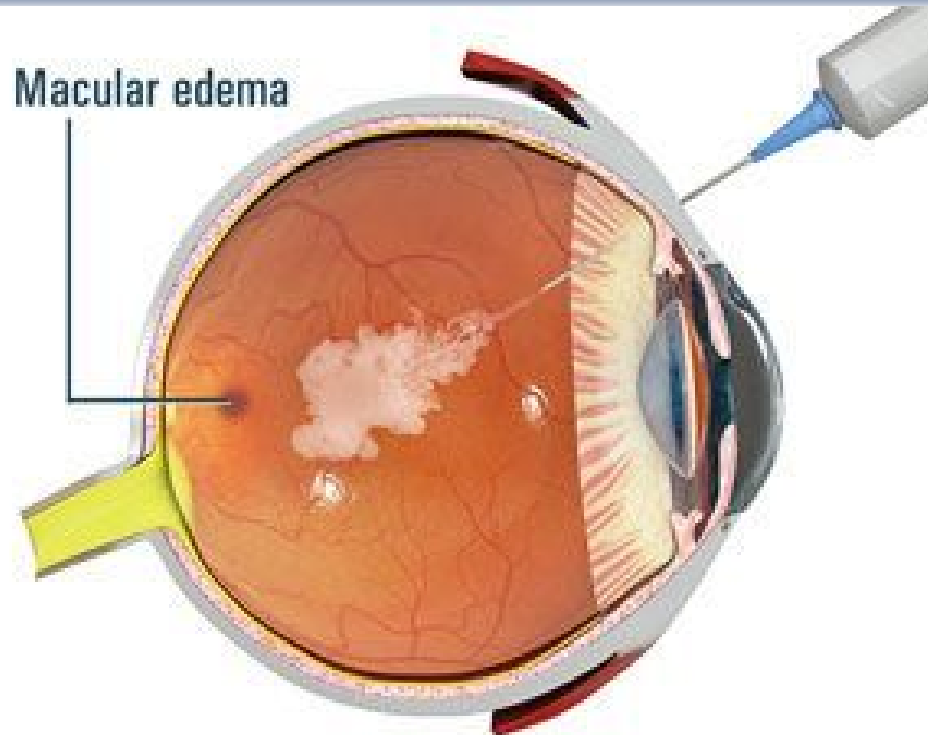
Subcutaneous

Ophthalmic

Otic

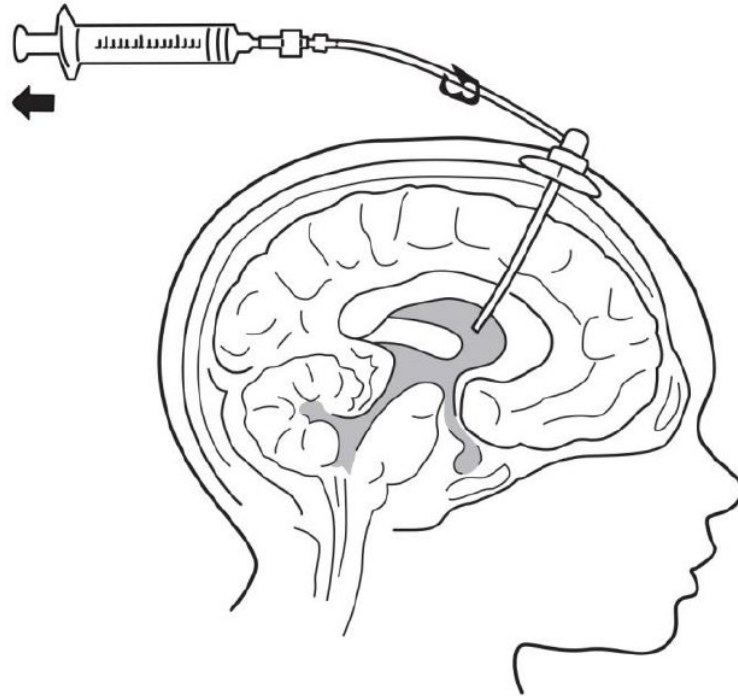
Complex Drug Delivery Methods

- Intravitreal Injection [e.g., aflibercept (Eylea®)]



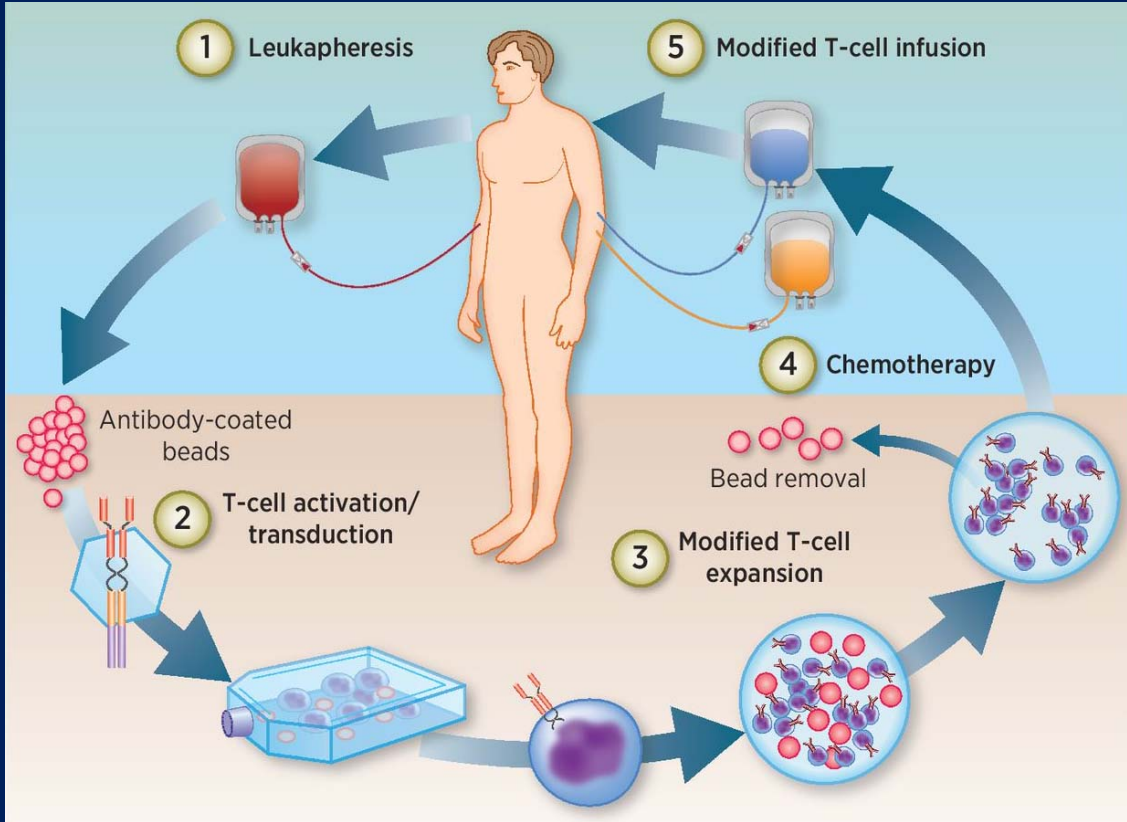
Complex Drug Delivery Methods

- Intraventricular Injection [e.g., cerliponase alfa (Brineura®)]



Complex Drug Delivery Methods

- Chimeric Antigen Receptor (CAR)-T Therapy



Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Rare and/or Complex Diseases

Cancers

Hemophilia

Cystic fibrosis

Multiple
sclerosis

Pulmonary
fibrosis

Hereditary
angioedema

Pulmonary
hypertension

Hutchinson-
Gilford Progeria

Neuromyelitis
Optica
Spectrum
Disorder

Prevalent Diseases with Specialty Drugs

Gout

Asthma

Hepatitis C

High
cholesterol

Atopic
dermatitis

Plaque
psoriasis

Rheumatoid
arthritis

Substance
use disorder

Orphan Drugs

- Used for very rare conditions
 - “Orphan diseases” affect < 200,000 patients
- Few or no therapeutic alternatives
- Examples
 - Lomitapide (Juxtapid[®]) for homozygous familial hypercholesterolemia
 - Elexacaftor/tezacaftor/ivacaftor (Trikafta[®]) for cystic fibrosis
 - Aripiprazole (Abilify[®]) for Tourette’s syndrome

Orphan Drug Designation

- Type of exclusivity offered by the FDA upon drug approval

Patent and Exclusivity for: N021436

Product 006
ARIPIPIRAZOLE (ABILIFY) TABLET 2MG

Exclusivity Data

Product No	Exclusivity Code	Exclusivity Expiration
006	ODE-80	12/12/2021

Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- **Clinical support essential to effective outcome**
- Reimbursement complexities
- High cost

Necessary Clinical Support

Timely and accurate fulfillment of prescriptions

Disease specific education

Adherence assistance

Follow-up between office visits

Management of adverse reactions, interactions

Specialty Pharmacies

- Focus on the proper dispensing of specialty drugs
- Owned by
 - Health plans
 - Pharmacy benefit managers
 - Retail/community pharmacies
 - Wholesalers
 - Private owners
- Most dispensing is done through mail

Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Customized dosing
- Complex delivery methods
- Chronic, complex, and/or rare disease target
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Reimbursement Complexities

Drugs picked up at a pharmacy

- Reimburse the pharmacy

Drugs administered by a provider

- Reimburse the doctor

Drugs bought at the pharmacy & administered by a provider

- Reimburse...?

Characteristics of Specialty Drugs

- Manufactured through a biotechnology process
- Special handling requirements
- Chronic, complex, and/or rare disease target
- Customized dosing
- Complex delivery methods
- Clinical support essential to effective outcome
- Reimbursement complexities
- High cost

Cost of Specialty Medications

- Average \$79,000 per year
- Nearly half of all drug spending
 - Only 2% of the population
- Specialty defined as cost > \$670 per month for Medicare

Sales Forecasts

Projected Rank	Generic Name	Brand Name	Forecasted Sales
1	Adalimumab	Humira	\$20 bn
2	Pembrolizumab	Keytruda	\$16.8 bn
3	Lenalidomide	Revlimid	\$12.7 bn
4	Apixaban	Eliquis	\$10.5 bn
5	Aflibercept	Eylea	\$8.9 bn
6	Nivolumab	Opdivo	\$8.8 bn
7	Ustekinumab	Stelara	\$8.4 bn
8	Bictegravir, emtricitabine, and tenofovir alafenamide	Biktarvy	\$8.4 bn
9	Ibrutinib	Imbruvica	\$7.6 bn
10	Rivaroxaban	Xarelto	\$7.6 bn

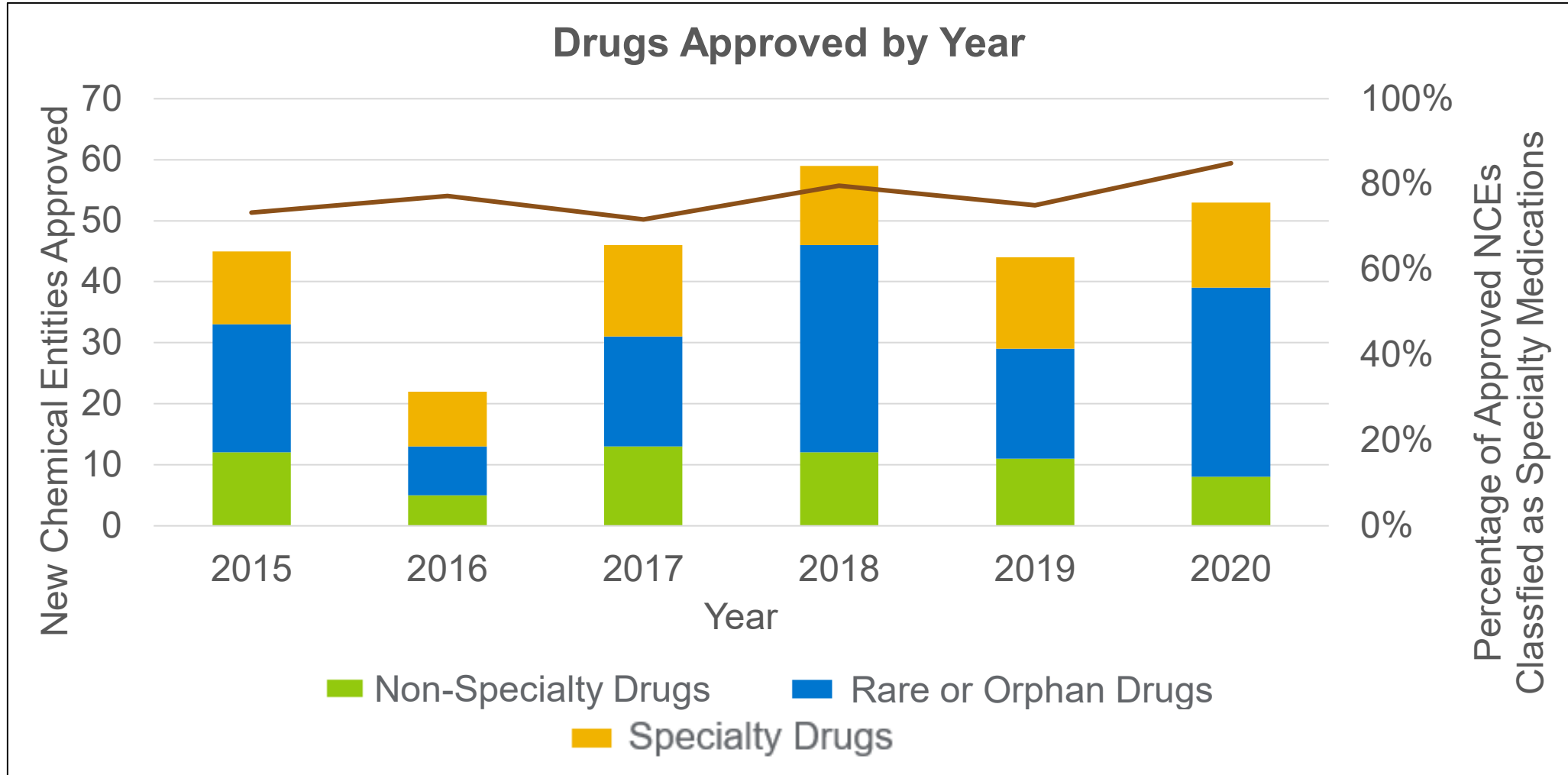
Sales Forecasts

Projected Rank	Generic Name	Brand Name	Forecasted Sales
1	Adalimumab	Humira	\$20 bn
2	Pembrolizumab	Keytruda	\$16.8 bn
3	Lenalidomide	Revlimid	\$12.7 bn
4	Apixaban	Eliquis	\$10.5 bn
5	Aflibercept	Eylea	\$8.9 bn
6	Nivolumab	Opdivo	\$8.8 bn
7	Ustekinumab	Stelara	\$8.4 bn
8	Bictegravir, emtricitabine, and tenofovir alafenamide	Biktarvy	\$8.4 bn
9	Ibrutinib	Imbruvica	\$7.6 bn
10	Rivaroxaban	Xarelto	\$7.6 bn

High Cost Specialty Drugs

Rank	Generic Name	Brand Name	Use	Annual Cost of Therapy
1	Onasemnogene abeparvovec	Zolgensma	Spinal muscular atrophy	\$2,125,000
2	Metreleptin	Myalept	Lipodystrophy	\$855,678
3	Voretigene neparvovec	Luxturna	Retinal dystrophy	\$850,000
4	Pralatrexate	Folotyn	T-cell lymphoma	\$793,870
5	Cerliponase alfa	Brineura	Neuronal ceroid lipofuscinosis type 2	\$716,040

Specialty Drug Approvals



Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Formulary Design

Specialty tier

Coinsurances versus copays

- Average coinsurance is 26%

Maximum out-of-pocket limits

Preferred and non-preferred specialty tiers

Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Pharmacy or Medical Benefits

- Different schools of thought
 - All drugs under pharmacy benefit
 - Provider-administered drugs under the medical benefit
- Benefit design strategies
 - Prevent pharmacy premium increases
 - Coverage may differ by benefit

Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Utilization Management

Prior
Authorization

Quantity
Limits

Authorization
Duration

Biosimilars

- Typical generics show bioequivalence
- Biosimilars show no meaningful difference in safety, potency, purity
 - “Highly similar” to an FDA-approved biologic product
 - Not generics, not interchangeable
- Biosimilar naming
 - Unique proprietary name
 - Meaningless suffix added to the nonproprietary name

Biosimilars

- Expected cost savings
 - 40-95% cost reduction for generics
 - 25-40% cost reduction for biosimilars
- Uptake limited by
 - Inability to automatically substitute
 - Different biosimilars covered by different plans
 - Prescriber hesitancy, lack of awareness
 - Patent litigation and other manufacturer interference

Approved Oncology Biosimilars

Originator	Nonproprietary Name	Biosimilar Suffix	Biosimilar Brand Name
Neupogen	filgrastim	-sndz -aafi	Zarxio Nivestym
Neulasta	pegfilgrastim	-cbqv -jmdb -bmez -apgf	Udenyca Fulphila Ziextenzo Nyvepria
Epogen	epoetin alfa	-epbx	Retacrit
Avastin	bevacizumab	-awwb -bvzr	Mvasi Zirabev
Herceptin	trastuzumab	-dkst -pkrb -dttb -anns -qyyp	Ogivri Herzuma Ontruzant Kanjinti Trazimera

Other Approved Biosimilars

Originator	Nonproprietary Name	Biosimilar Suffix	Biosimilar Brand Name
Rituxan	rituximab	-abbs -pvvr -arrx	Truxima Ruxience Riabni
Remicade	infliximab	-dyyb -abda -qbtx -axxq	Inflectra Reflexis Ixifi Avsola
Enbrel	etanercept	-szzs -ykro	Erelzi Eticovo
Humira	adalimumab	-atto -adbm -adaz -afzb -bwwd -fkjp	Amjevita Cyltezo Hyrimoz Abrilada Hadlima Hulio

Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Copay Card Programs

- Managed care organizations hate copay cards
- Copay accumulator programs
 - Copay cards no longer count toward deductibles and max out-of-pocket limits
- Copay maximizer programs
 - Maximum value of copay card is applied evenly throughout the year

Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- **Contracting**
- Case and disease management

Contracting

Specialty pharmacy contracting

- Performance guarantees
- Narrow networks, limited distribution drugs

Manufacturer contracting

- Done on non-specialty medications as well
- Outcomes-based or value-based contracts

Site of care strategies

- Home health
- Specific sites

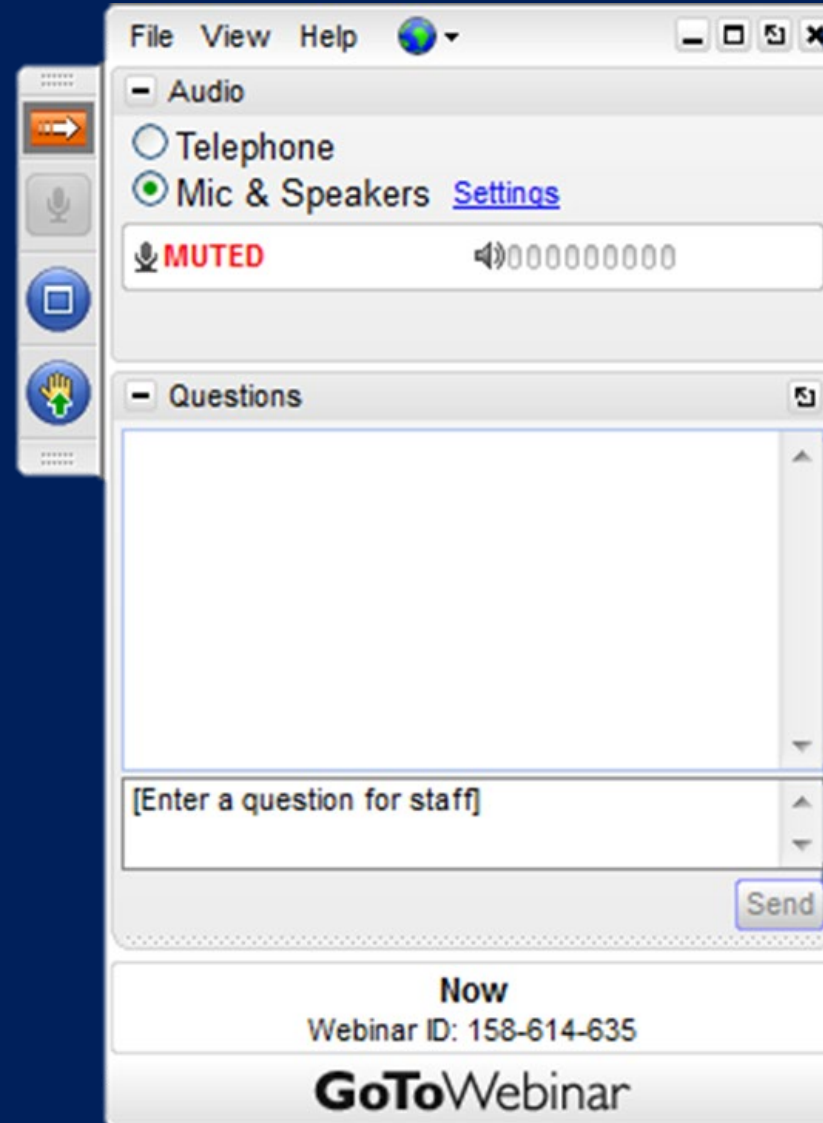
Specialty Trend Management

- Formulary design
- Pharmacy versus medical benefits
- Utilization management
- Copay card programs
- Contracting
- Case and disease management

Conclusion

- No universal definition
- Increasing number of high cost specialty medications
- Variety of trend management strategies

How to Ask a Question



**For a list of upcoming webinars,
visit www.amcp.org/calendar**

References

- Barone J, Jardina PE. New therapies and the role of specialty pharmacy providers. Pharmacy Times. 2018. Available at: <https://www.pharmacytimes.com/publications/specialty-pharmacy-times/2018/January-2018/New-Therapies-and-the-Role-of-Specialty-Pharmacy-Providers>
- Brineura [prescribing information]. Novato, CA. BioMarin Pharmaceutical Inc.; 2020.
- Chase L. The 10 most expensive drugs in the U.S., period. GoodRx. Available at: <https://www.goodrx.com/blog/most-expensive-drugs-period/>
- Collins F. FDA approves first CAR-T cell therapy for pediatric acute lymphoblastic leukemia. 2017. Available at: <https://directorsblog.nih.gov/2017/08/30/fda-approves-first-car-t-cell-therapy-for-pediatric-acute-lymphoblastic-leukemia/>
- Express Scripts. 2019 drug trend report. 2020. Available at: <https://www.express-scripts.com/corporate/drug-trend-report>
- Hahn SM. Rare disease day 2020: FDA continues important work on treatments for rare diseases. U.S. Food and Drug Administration. 2020. Available at: <https://www.fda.gov/news-events/fda-voices/rare-disease-day-2020-fda-continues-important-work-treatments-rare-diseases>
- Imlygic [prescribing information]. Thousand Oaks, CA: BioVex, Inc.; 2019.
- iPharmaCenter. Expected best selling pharmaceutical products (drugs) in 2021. 2021. Available at: <https://www.ipharmacenter.com/post/expected-best-selling-pharmaceutical-products-drugs-in-2021>
- Joachim R. Weighing the potential of Humira biosimilars in the U.S. – Competitive dynamics analysis. Biosimilar Development. 2020. Available at: <https://www.biosimilardevelopment.com/doc/weighing-the-potential-of-humira-biosimilars-in-the-u-s-competitive-dynamics-analysis-0001>

References

- Kymriah [prescribing information]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; 2020.
- Li E, Ramanan S, Green L. Pharmacist substitution of biological products: issues and considerations. *J Manag Care Spec Pharm.* 2015;21(7):532-539.
- Schondelmeyer SW, Purvis L. Trends in retail prices of prescription drugs widely used by older Americans: 2017 year-end update. AARP Public Policy Institute. 2019. Available at: <https://www.aarp.org/content/dam/aarp/ppi/2019/09/trends-in-retail-prices-of-prescription-drugs-widely-used-by-older-americans.doi.10.26419-2Fppi.00073.003.pdf>
- U.S. Food and Drug Administration. Biosimilar product information. 2020. Available at: <https://www.fda.gov/drugs/biosimilars/biosimilar-product-information>
- U.S. Food and Drug Administration. Generic competition and drug prices. 2019. Available at: <https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/generic-competition-and-drug-prices>
- U.S. Food and Drug Administration. New Drug Therapy Approvals 2020. 2021. Available at: <https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/new-drug-therapy-approvals-2020>
- U.S. Food and Drug Administration. Nonproprietary naming of biological products – Guidance for industry. 2017. Available at: <https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM459987.pdf>
- U.S. Food and Drug Administration. Orange book: approved drug products with therapeutic equivalence evaluations. <https://www.accessdata.fda.gov/scripts/cder/ob/index.cfm>

References for Images

- GalChimia. Are cancer immunotherapy and small molecules compatible? 2018. Available at: <https://www.galchimia.com/are-cancer-immunotherapy-and-small-molecules-compatible/>
- Amgen. Imlygic clinical overview and handling guide. 2019. Available at: <https://cdn.imlygichcp.com/cdn/917dac5d-ff46-4382-bb1e-8a4041ae951b/en/1/20201222t152404z/imlygic-clinical-overview.pdf>
- Clinical OMIC. FDA Approves Novartis' Kymriah As First CAR-T Cancer Immunotherapy. 2017. Available at: <https://www.clinicalomics.com/topics/oncology/fda-approves-novartis-kymriah-as-first-car-t-cancer-immunotherapy/>
- North Shore Eye Centre. Intravitreal triamcinolone. Available at: <http://www.northshoreeye.com.au/intravitreal-triamcinolone/>
- ThermoFisher Scientific. A huge step forward for immunotherapy: FIRST ever FDA approved CAR-T cell therapy. 2017. Available at: <https://www.thermofisher.com/blog/behindthebench/a-huge-step-forward-for-immunotherapy-first-ever-fda-approved-car-t-cell-therapy/>