# San Francisco Health Service System
# Health Service Board

## Genomics and Pharmacy High-Cost Drugs

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# Introduction

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# Agenda

## Past, Present and Future

- Drug Manufacturer Strategy
- Recent FDA Approvals

- Market Outlook
- Rare Disease

- Specialty Drug Expansion
- Solutions
- Legislative Efforts

# 1

# Drug Manufacturer Strategy and Recent FDA Approvals

# Drugs to Market

## Manufacturer Strategy

### Novel Drug Approvals

- Novel drugs are innovative products that serve previously unmet medical needs or otherwise significantly help to advance patient care and public health.
- Going through the FDA approval process results in approval, Complete Response Letters, or Refuse to File Letters.
- As of July 2022, the FDA has issued a total of 6 Complete Response letters and Refuse to File letters.

### Expanding Indications

- Manufacturers will implement life cycle management. Life cycle management is asking, “what more can this drug do?”
- Life cycle management expands a drug’s indication to different disease or therapeutic areas.
- Expanding indication requires additional FDA approval.

### New Routes of Administration

- Can this drug be delivered more conveniently/efficiently to increase patient outcomes and minimize side effects?
- Examples include taking a medication that was administered in clinic via intravenous infusion to a self-administered subcutaneous injection.

# FDA Novel Drug Approvals for 2022

Current as of July 2022

## Approvals

16

FDA has approved 16 Novel Drugs as of July 2022

## Therapeutic Area

7

The 16 drugs span across 7 different therapeutic areas

## Rare Disease

7/16

7 of the 16 drugs approved treat rare or genetic diseases

## Expenditure

$1,000+

Average cost for one month, climbing upwards of $100,000 + for a year of treatment

| DRUG NAME | ACTIVE INGREDIENT | APPROVAL DATE | FDA-APPROVED USE ON APPROVAL DATE* |
|---|---|---|---|
| Amvuttra | Vutrisiran* | 6/13/2022 | To treat polyneuropathy of hereditary transthyretin-mediated amyloidosis |
| Vtama | Tapinarof | 5/23/2022 | To treat plaque psoriasis |
| Mounjaro | Tirzepatide | 5/13/2022 | To improve blood sugar control in diabetes, in addition to diet and exercise (Press Release) |
| Voquezna | Vonoprazan, amoxicillin, and clarithromycin | 5/3/2022 | To treat Helicobacter pylori infection |
| Camzyos | Mavacamten* | 4/28/2022 | To treat certain classes of obstructive hypertrophic cardiomyopathy |
| Vivjoa | Oteseconazole | 4/26/2022 | To reduce the incidence of recurrent vulvovaginal candidiasis (RVVC) in females with a history of RVVC who are not of reproductive potential |
| Pluvicto | Lutetium (177Lu) vipivotide tetraxetan | 3/23/2022 | To treat prostate-specific membrane antigen-positive metastatic castration-resistant prostate cancer following other therapies |
| Opdualag | Nivolumab and relatlimab-rmbw | 3/18/2022 | To treat unresectable or metastatic melanoma |
| Ztalmy | Ganaxolone* | 3/18/2022 | To treat seizures in cyclin-dependent kinase-like 5 deficiency disorder |
| Vonjo | Pacritinib* | 2/28/2022 | To treat intermediate or high-risk primary or secondary myelofibrosis in adults with low platelets |
| Pyrukynd | Mitapivat* | 2/17/2022 | To treat hemolytic anemia in pyruvate kinase deficiency |
| Enjaymo | Sutimlimab-jome* | 2/4/2022 | To decrease the need for red blood cell transfusion due to hemolysis in cold agglutinin disease |
| Vabysmo | Faricimab-svoa | 1/28/2022 | To treat neovascular (wet) aged-related macular degeneration and diabetic macular edema |
| Kimmtrak | Tebentafusp-tebn* | 1/25/2022 | To treat unresectable or metastatic uveal melanoma |
| Cibinqo | Abrocitinib | 1/14/2022 | To treat refractory, moderate-to-severe atopic dermatitis |
| Quviviq | Daridorexant | 1/7/2022 | To treat insomnia |

# 2

# Drug Approval Pipeline and Market Outlook

# FDA Pipeline Agents 2022 – 2023

## NDA/BLA Submissions Under Review


## Future Filing Dates


## Cost Implications

- 26% of pipeline agents are used to treat rare diseases
- 49% have orphan drug designation
- Orphan drugs are 25 times more costly than non-orphan drugs
- 88% of orphan drugs cost greater than $10,000 per patient per year

# Deeper Dive into Rare Disease

## Emergence of Gene and Cell Therapies

- Gene therapy is introducing exogenous gene(s) into the body. This may be to supply a missing gene, bypass role of a missing gene, or augment therapy for a disease. These are costly treatments to cure rare and fatal diseases.
- There are currently 23 approved gene and cell therapies.
- There are approximately 7,000 rare diseases, affecting 25 to 30 million Americans (1 in 10 Americans).

# Cost Perspective

- Zolgensma, used to treat spinal muscular atrophy, costs $2.12M for a one-time dose.
- Luxturna, used for inherited retinal diseases, costs $850,000 for a one-time dose.
- Other very high-cost drugs launching in 2022 shown on next slide.
- Over 900 investigational drug applications waiting FDA approval.

# Gene Therapies

## Exponentially Growing

| Drug | U.S. Prevalence | Launch | Cost |
|---|---|---|---|
| Zolgensma | 1 in 10,000 live births | Available | $2.12M |
| Luxturna | 1,000 – 2,000 patients | Available | $850K |
| Upstaza | 1 in 90,000 live births | 2022 | $3.0M |
| Skysona | 1 in 21,000 male births | 2022 | $2.5M |
| Roctavian | 18,000 patients | 2022 | $2.5M |
| AMT-061 | 6,000 patients | 2022 | $2.5M |
| Zyntenglo | 3,000 patients | 2022 | $1.85M |

## Market Growth

- Gene therapy market size valued at $4.99B in 2021
- Market size expected to grow to $36.2B by 2027
- FDA expected to approve 10 to 20 products per year in gene and cell therapy market by 2025

# 3

# Growth of Specialty Medications and Payer Response

# Expansion of Specialty Medications

## Examining Data and Trend

- Specialty market in 2020 was $254B. In 2025, the specialty market is expected to be $373B.
- In 2010, specialty medications accounted for 26% of spend. Specialty medications now account for 53% of spend.
- 500 new drugs are estimated to seek FDA approval in the next 3 years.
  - 67% of those drugs are specialty medications.
- Within the next 3 years, the FDA is expected to approve up to 20 gene and cell therapies.
- Therapeutic areas that are driving the growth of specialty medications are autoimmune and oncology

**Growth**

$373B

Specialty drug market is predicted to be by 2025

**Expenditure**

53%

Specialty spend with respect to total prescription spend

**Specialty Medications**

67%

Seeking FDA Approval in next 3 years

**Gene and Cell Therapies**

20

FDA approved therapies by 2025

# Solutions

Pharmacy Benefit Manager (PBM) and Payer Response

- **Value-Based Contracting** → Agreement made between PBM and drug manufacturer. If drug does not work for patient, manufacturer will reimburse PBM for cost of medication
- **Drug Management Services** → Tier adjustments and clinical concierge services for patients on orphan drugs or high-cost medications
- **Installment Payment Plan** → Mitigate immediate financial impact; paid over several years
- **Pooled PMPM Programs** → Programs that protect payers and employers from gene therapy associated high-cost claims

# Biosimilars

## “Generics” of Branded Biologics

- A copy of a biologic medicine, but not identical
- No clinically meaningful differences in terms of safety and effectiveness from reference product

- Comprehensive evaluation of analytic, animal, pharmacology and clinical studies
- May differ in clinically inactive components, indications and product forms

- Biosimilars have an “easier” pathway to approval when compared to branded biologics
- Projected to provide a 30% discount — reducing spend by $100B over the next 5 years

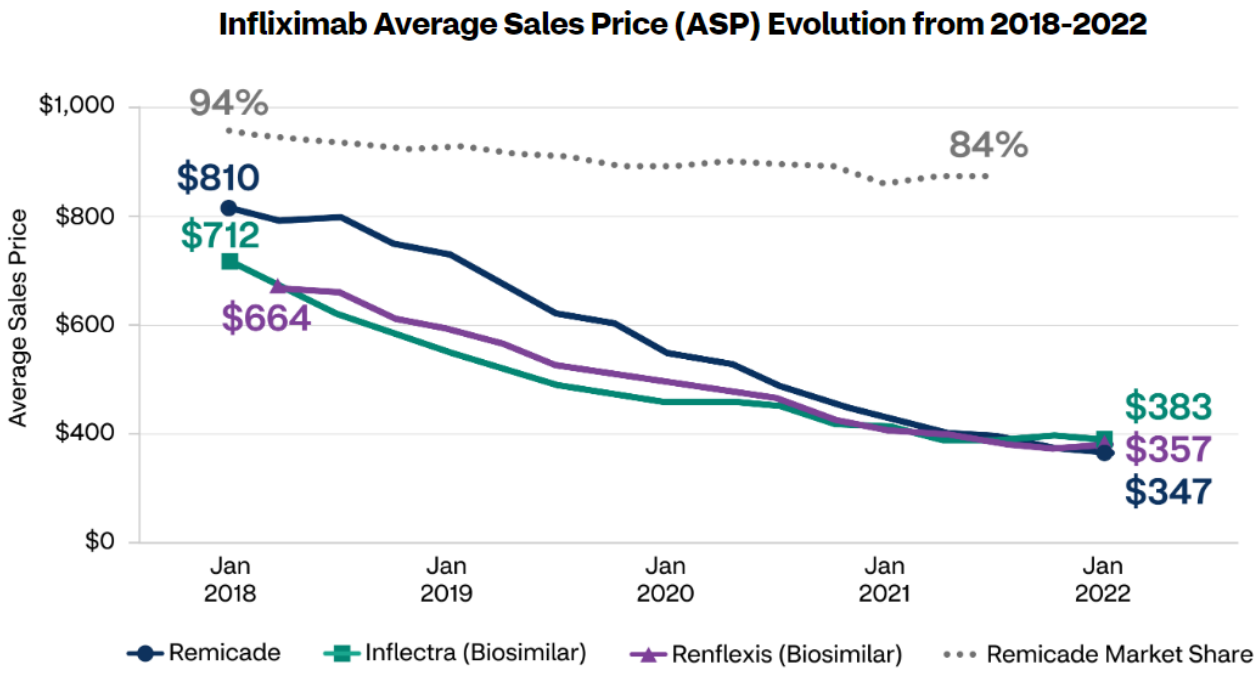
Biosimilar Future Opportunity

**$49B** OPPORTUNITY | **76** PATENT EXPIRATIONS THROUGH 2020 – 2024

# Biosimilars

## Driving Cost Down

- Biosimilars increase drug marketplace competition, making costly medications more affordable.
- The introduction of Infliximab biosimilars demonstrate marketplace competition, cutting drug cost by over 50%.
- Almost all biosimilars have launched at a WAC of 3% to 24% below the reference product ASP.
- With more biosimilar approvals, we see increased biosimilar utilization. Therapeutic areas such as oncology have seen growth of 79% from 2016 to 2021.
- Currently, there are 36 approved biosimilars and 21 launched biosimilars



# Newsom's CalRx Biosimilar Insulin

## Affordable Insulin

| Contracting | Budget | Manufacturing | Legislation |
|---|---|---|---|
| • State will be working directly with CMO<br>• Partnering with CMO is more cost effective in comparison to state manufacturing insulin itself | • $50M used to the development of biosimilar insulin products in vial and pen formulations<br>• $50M used for in-state manufacturing facility | • "At least one form of insulin"<br>• $50M for development used towards an interchangeable biosimilar insulin product | • Affordable Insulin Now Act<br>• Efforts to cap out-of-pocket costs at $35 per month for Medicare and private insurance<br>• Must pass senate |

# Prescription Drug Provisions in the Senate Reconciliation Proposal

## Efforts to Reduce Prescription Cost

| Purpose | Implementation | Key Provisions |
| --- | --- | --- |
| • Reduce prescription drug cost for Medicare beneficiaries<br>• Limit increases in drug prices for Medicare and private insurance | • Aims to reduce federal deficit by $288B over 10 years<br>• Requires drug manufacturers to pay rebates if drug cost greater than inflation<br>• Implementing cost saving measures: Out-of-pocket maximum, catastrophic coverage, expanding eligibility | • Enables federal government to negotiate prices for high-cost medications for Medicare<br>• Improved rebates for drugs impacted by inflation<br>• Repeal Trump Administrations drug rebate rule |

# Pharmacy Benefit Manager (PBM) Transparency Act

## PBM Reform

| Purpose | Implementation | Key Provisions |
|---|---|---|
| • Reduce prescription drug cost for Medicare beneficiaries<br>• Limit increases in drug prices for Medicare and private insurance | **Spread Pricing**<br>• PBMs can no longer charge a health plan/payer a different amount than what they reimburse a pharmacy for (retaining the difference).<br>**Claw back**<br>• PBMs can no longer reduce or rescind reimbursements paid to a pharmacy for a prescription. | • PBMs would be required to annually report multiple indicators to the FTC<br>• Spread pricing, fees, formulary changes, reimbursements, etc.<br>• Violation of the act may result in $1,000,000 penalty, including civil action<br>• Must pass senate |

# Questions