San Francisco Health Service System
Health Service Board

Genomics and Pharmacy High-Cost Drugs

August 11, 2022
Introduction

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Health Solutions

Agenda
Past, Present and Future

- Drug Manufacturer Strategy
- Recent FDA Approvals
- Market Outlook
- Rare Disease
- Specialty Drug Expansion
- Solutions
- Legislative Efforts
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**

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

- **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

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HSB Meeting: Genomics and Pharmacy High-Cost Drugs — August 11, 2022
Drug Approval Pipeline and Market Outlook
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

NDA/BLA Submissions Under Review

- 45 Agents Under Review
- 18 Therapeutic Areas
- 7 Routes of Administration

Future Filing Dates

- 20 Agents Under Review
- 12 Therapeutic Areas
- 6 Routes of Administration
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

<table>
<thead>
<tr>
<th>Drug</th>
<th>U.S. Prevalence</th>
<th>Launch</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td>Zolgensma</td>
<td>1 in 10,000 live births</td>
<td>Available</td>
<td>$2.12M</td>
</tr>
<tr>
<td>Luxturna</td>
<td>1,000 – 2,000 patients</td>
<td>Available</td>
<td>$850K</td>
</tr>
<tr>
<td>Upstaza</td>
<td>1 in 90,000 live births</td>
<td>2022</td>
<td>$3.0M</td>
</tr>
<tr>
<td>Skysona</td>
<td>1 in 21,000 male births</td>
<td>2022</td>
<td>$2.5M</td>
</tr>
<tr>
<td>Roctavian</td>
<td>18,000 patients</td>
<td>2022</td>
<td>$2.5M</td>
</tr>
<tr>
<td>AMT-061</td>
<td>6,000 patients</td>
<td>2022</td>
<td>$2.5M</td>
</tr>
<tr>
<td>Zyntenglo</td>
<td>3,000 patients</td>
<td>2022</td>
<td>$1.85M</td>
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### 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
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

<table>
<thead>
<tr>
<th>Contracting</th>
<th>Budget</th>
<th>Manufacturing</th>
<th>Legislation</th>
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<tbody>
<tr>
<td>• State will be working directly with CMO</td>
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<tr>
<td>• Partnering with CMO is more cost effective in comparison to state manufacturing insulin itself</td>
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<td>• $50M used to the development of biosimilar insulin products in vial and pen formulations</td>
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<tr>
<td>• $50M used for in-state manufacturing facility</td>
<td></td>
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<tr>
<td>• “At least one form of insulin”</td>
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<tr>
<td>• $50M for development used towards an interchangeable biosimilar insulin product</td>
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<tr>
<td>• Affordable Insulin Now Act</td>
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<tr>
<td>• Efforts to cap out-of-pocket costs at $35 per month for Medicare and private insurance</td>
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<td>• Must pass senate</td>
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# Prescription Drug Provisions in the Senate Reconciliation Proposal

## Efforts to Reduce Prescription Cost

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<tr>
<th>Purpose</th>
<th>Implementation</th>
<th>Key Provisions</th>
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</thead>
<tbody>
<tr>
<td>• Reduce prescription drug cost for Medicare beneficiaries</td>
<td>• Aims to reduce federal deficit by $288B over 10 years</td>
<td>• Enables federal government to negotiate prices for high-cost medications for Medicare</td>
</tr>
<tr>
<td>• Limit increases in drug prices for Medicare and private insurance</td>
<td>• Requires drug manufacturers to pay rebates if drug cost greater than inflation</td>
<td>• Improved rebates for drugs impacted by inflation</td>
</tr>
<tr>
<td></td>
<td>• Implementing cost saving measures: Out-of-pocket maximum, catastrophic coverage, expanding eligibility</td>
<td>• Repeal Trump Administrations drug rebate rule</td>
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</table>
# Pharmacy Benefit Manager (PBM) Transparency Act

## PBM Reform

<table>
<thead>
<tr>
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<th>Implementation</th>
<th>Key Provisions</th>
</tr>
</thead>
</table>
| • Reduce prescription drug cost for Medicare beneficiaries  
• Limit increases in drug prices for Medicare and private insurance | **Spread Pricing**  
• PBMs can no longer charge a health plan/payer a different amount than what they reimburse a pharmacy for (retaining the difference).  
**Claw back**  
• 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  
• Spread pricing, fees, formulary changes, reimbursements, etc.  
• Violation of the act may result in $1,000,000 penalty, including civil action  
• Must pass senate |
Questions