

ASHP Virtual Roundtable Series: Next Generation Therapeutics

Coordinated with the ASHP Center on Next Generation Therapeutics & the Sections of Specialty Pharmacy Practitioners and Clinical Specialists and Scientists

Case Studies

Case Presenters

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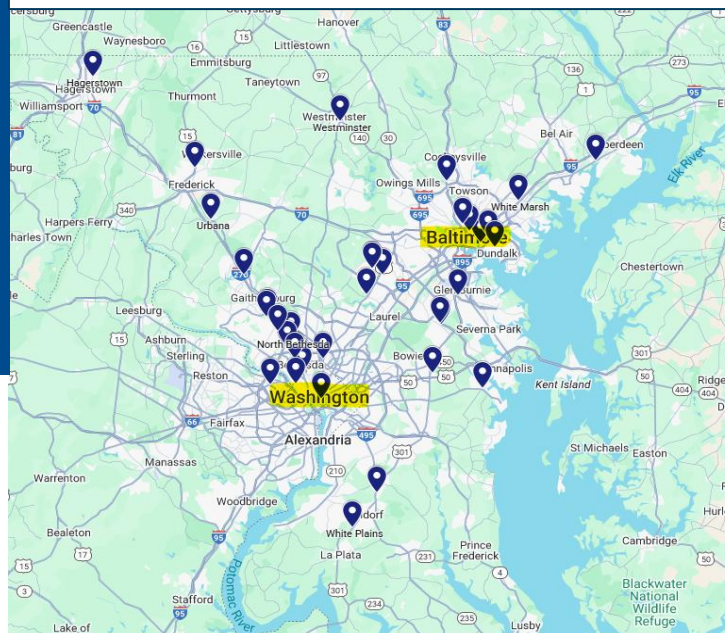
Case Study 1: Organizational structures and governance

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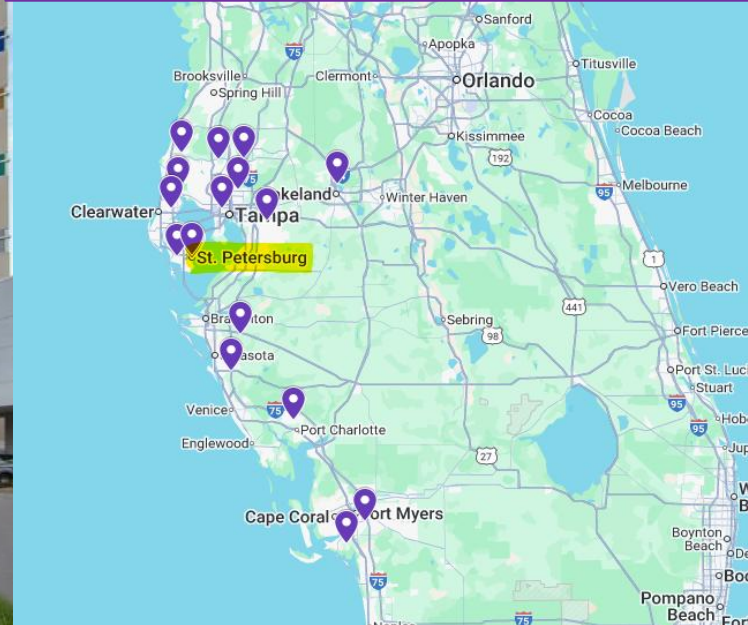


JOHNS HOPKINS MEDICINE

Baltimore, MD – Washington, D.C.



St. Petersburg, FL



- Located in Baltimore, MD, Washington, D.C., and St. Petersburg, FL metropolitan areas
- 6 hospitals (3 academic medical centers, 3 community hospitals)
- 50+ outpatient specialty and primary care sites
- 16 ambulatory infusion suites
- 10 community/specialty pharmacy locations
- 7 ambulatory surgery centers
- 2 Hemophilia Treatment Centers
- Johns Hopkins Care at Home (full-service home care provider)
- Johns Hopkins Health Plans
- Johns Hopkins Medicine International
- 40,000+ employees

Cell and Gene Therapy (CGT) Program Overview

- Program Established: 2018
- Current locations:
 - The Johns Hopkins Hospital / Johns Hopkins Care at Home (Baltimore, MD)
 - Johns Hopkins All Children's Hospital (St. Petersburg, FL)
 - Sibley Memorial Hospital (Washington, D.C)
- Therapies offered:
 - Oncology: CAR-T, TIL
 - In vivo gene therapies: Adstiladrin, Elevidys, Hemgenix, Roctavian, Zolgensma
- Onboarding underway: Ryoncil, Papzimeos, Encelto, Casgevy, Lyfgenia

Foundational CGT Infrastructure Domains

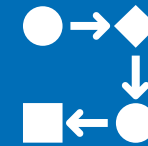
Financial



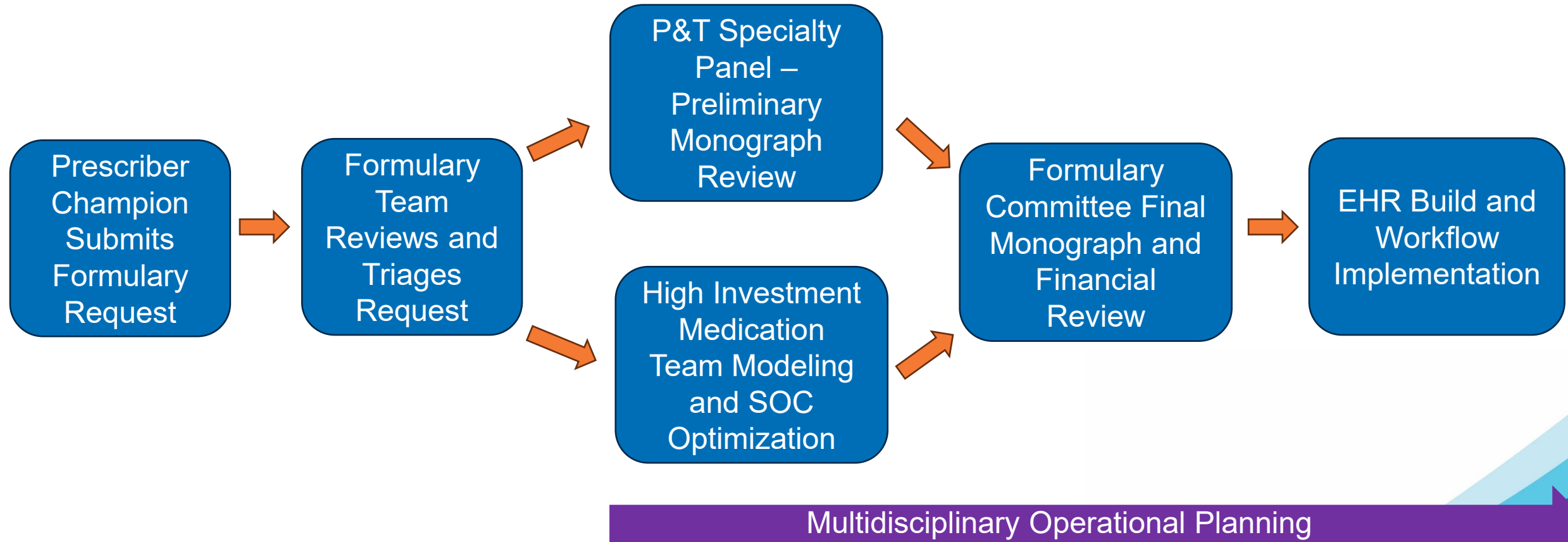
Clinical



Operational



Formulary and High-Cost Drug Review



CGT Program Arms

RESEARCH

**CELLULAR
THERAPY /
CAR-T**

GENE THERAPY

CAR-T / Cell Therapy Program Governance

- Steering committee is primarily led by Oncology Center administrators, physicians, APP, nursing, and cell therapy lab leadership – manage clinical, operational, and financial domains
- Pharmacy is involved with:
 - Core clinical workgroup: clinical guideline and QI development
 - Treatment plan consulting and dose adjustments (e.g., lymphodepletion and toxicity management)
- Pharmacy is NOT involved with:
 - Patient identification and selection
 - Financial clearance and RCM
 - Patient education on treatment journey

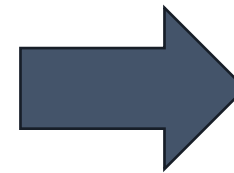
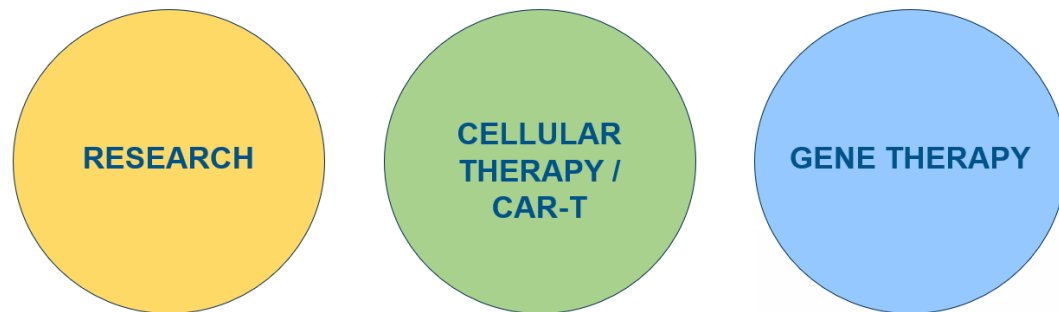
Gene Therapy Program Governance

- Overarching governance structure is in the works, but starting off with Neuromuscular Physician Champion, Gene Therapy CRNP, and Gene Therapy Clinical Coordinator Pharmacist leading a multidisciplinary, system-wide Gene Therapy Clinical Group
- Pharmacy plays a central role in all gene therapy cases and leads coordination across the following work areas:
 - Patient selection and formulary criteria
 - Revenue Cycle Management support
 - Front-end: Prior auth, medication access, insurance contracting/SCA
 - Back-end: Claim submission and collection status updates
 - Case communication and executive approval
 - Procurement
 - Pharmacy receipt, storage/handling, and compounding/preparation
 - Administration instructions and nursing team support
 - Staff education and training
 - Workflow/SOP development

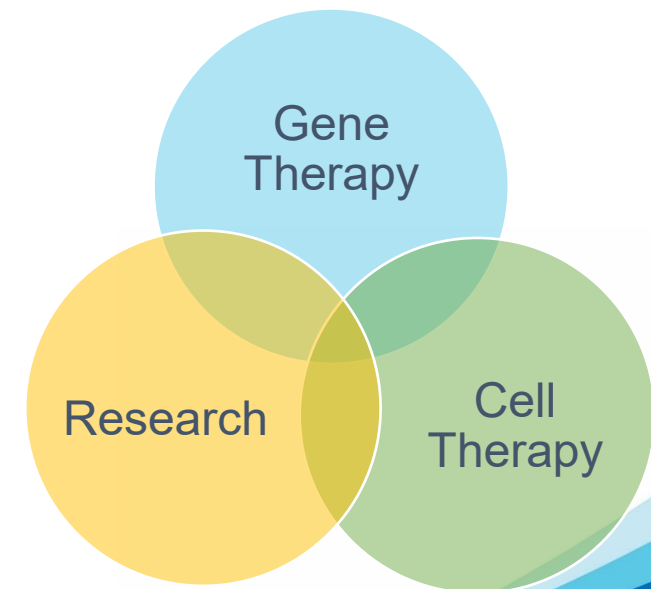
Future Directions

- Develop a cohesive strategy that bridges Research, Cell Therapy, and Gene Therapy programs to foster shared infrastructure and collaboration:

Current State



Future State



Future Directions

- Unify program arms under each domain to achieve alignment:



Case Study 2: Organizational structures and governance

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Vanderbilt University Medical Center (VUMC)

Pharmacy Services Overview

Hospitals

Main Campus,
Children's, and
Regionals

Clinics

Over 300 sites
across three
states

Retail

Four sites
supported by
Central Fill

Specialty

Limited
distribution
drugs (REMS)

Melrose Support Services Facility – Business Support Operations

- Central Fill
- Home Delivery
- Clinic Distribution
- Specialty Call Center
- Billing / Revenue Cycle
- Pharmacy Administration
- Vanderbilt Health Affiliated Network (VHAN) / Population Health Programs
- Product Packaging / Labeling / Storage
- Retail Support Group
- 340B Programs
- Technician Training Program
- Medication Cabinet Replenishment (Hospitals/Clinics)

Hospital and Clinic Revenue Cycle Support

Patient assistance program and foundation support

Medical prior authorizations, denials, and co-pay cards

Billing configurations and medication file setup

Waste billing

Price transparency

Medication cost updates

Charge review

High cost drugs/Expanded Review of Therapies (ERT)

Hospital and Clinic Revenue Cycle Support

Patient assistance program and foundation support

Medical prior authorizations, denials, and co-pay cards

Billing configurations and medication file setup

Waste billing

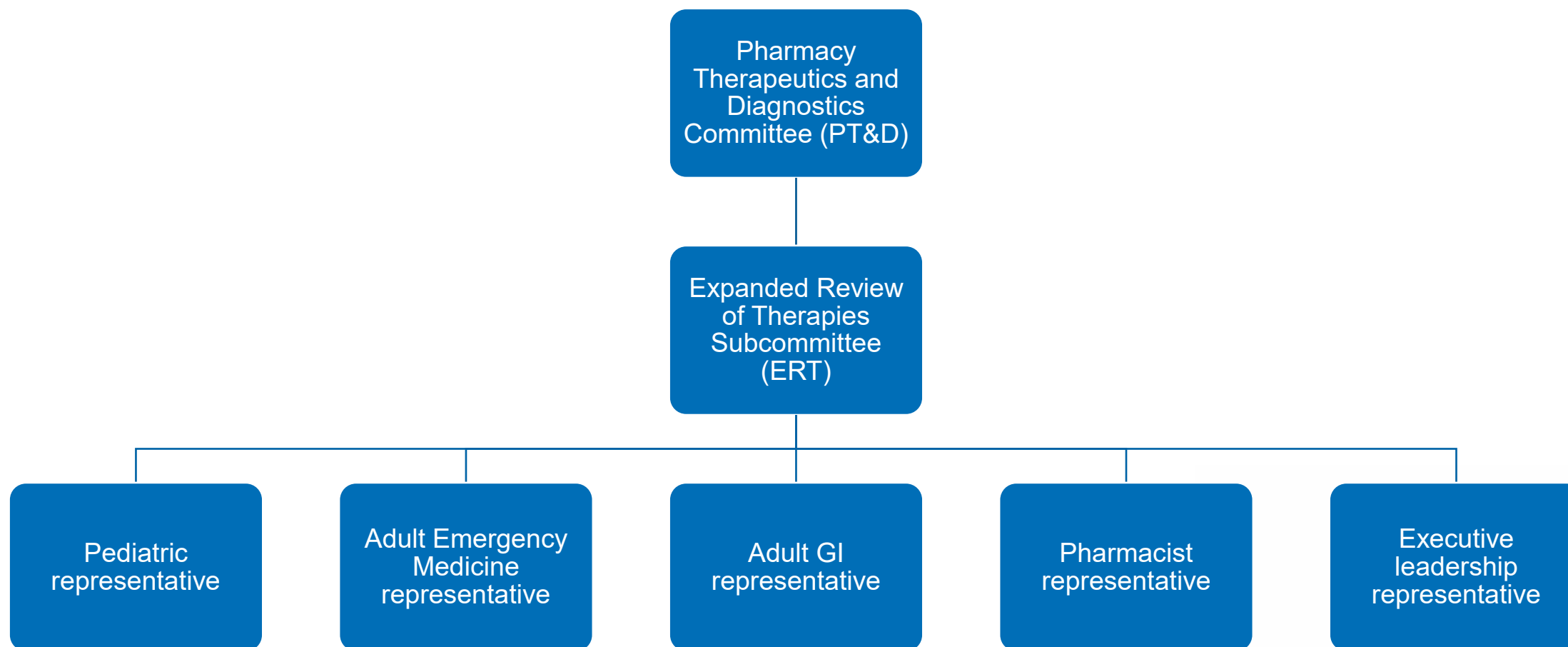
Price transparency

Medication cost updates

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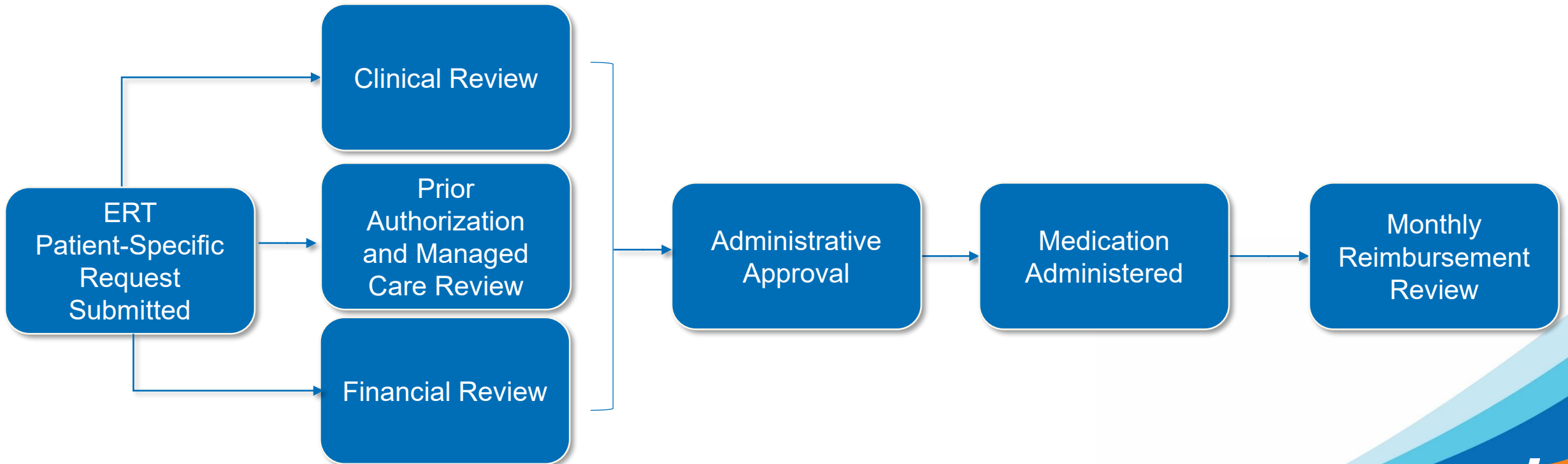
High cost drugs/Expanded Review of Therapies (ERT)

ERT Subcommittee Structure



VUMC ERT Process

- Medication cost \geq \$250,000 per year per patient
- Pharmacist coordinates review through collaboration with physician-led committee, finance, managed care, and PT&D



Medications with ERT Designation

Drug name	Pharmacologic Category
Abecma, Amtagvi, Aucatzyl, Breyanzi, Carvykti, Casgevy, Lyfgenia, Tecelra, Zynteglo	CAR-T therapy, T-cell therapy
Aldurazyme, Cerezyme, Elaprase, Elfabrio, Fabrazyme, Kanuma, Lumizyme, Naglazyme, Nexvazyme, VPRIV	Enzyme therapy
Elevidys, Evkeeza, Spinraza, Zolgensma	Rare disease, gene therapies
Anktiva, Beleodaq, Bizengri, Columvi, Danyelza, Elrexio, Emrelis, Fyarro, Papzimeos, Rytelo	Antineoplastic agents
Bizengri, Gamifant, Soliris, Ultomiris, Uplizna	Monoclonal antibodies

ERT Review Meetings

- **Weekly or Bi-Weekly**

- Elevidys workgroup
- Cell and gene therapy group (case conference, coordinator meeting)
- Additional stakeholder meetings (e.g. managed care, prior authorization and denial teams)

- **Monthly**

- Claim review of all high-cost therapy administrations
 - Additional therapies that undergo monthly high-cost claim review include Amvuttra, Elahere, Elzonris, Epkinly, Givlaari, Kimmtrak, Opdualag, Rybrevant, Sylvant, Talvey, Tecartus, Tecvayli, Tepezza, Tezspire, Tivdak, Vyvgart, and Yescarta
- Presentation and review with leadership

Program Director Responsibilities

- Conduct clinical reviews of ERT requests with ERT Subcommittee
- Facilitate prior authorization approvals with the clinical team
- Liaise with purchasing and clinical teams
- Collaborate with managed care team to assess single case agreement status
- Prepare financial estimates for review by finance and executive leadership
- Monitor high cost drug claims to ensure appropriate reimbursement

ERT Request Tracking

ERT Request Submissions

- Patient information
- Details about patient case and medication requested (dose, setting, anticipated duration, etc.)
- Prior authorization information
- Subcommittee approval
- Financial and leadership approval

ERT Request Tracker

- Patient information
- ERT number
- Medication requested
- Step in process
- Submission date
- Subcommittee approval and review information
- Insurance type
- PA and single case agreement status
- Financial and leadership review

Cellular Therapy Tracker

- Patient information
- Authorization information
- Managed care and leadership approval
- Purchasing information
- Post infusion verification
- Charge, invoice, and payment information

Lessons Learned

- **Transparency drives success!**
 - Clear, consistent, over-communication is key across all moving parts
- **Stakeholder engagement matters**
 - Consistent buy-in and routine quality improvement reviews strengthen program outcomes
- **Collaboration enables progress**
 - A consistent posture to align goals rather than create barriers
- **Adapt to ensure ongoing innovation**
 - Stay up to date with emerging therapies to proactively discuss workflows and anticipate barriers/challenges

Future Directions

- **Implement an institutional dashboard**
- **Solicit regular feedback regarding ERT process**
- **Develop standardized processes for unique requests**
- **Share best practices!**

Case Study 3: Strategic Partner and Internal Stakeholder Engagement

Ashley Dalton, PharmD, MHA
Associate Chief Pharmacy Officer
UC San Diego Health

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UC Health for Californians

EDUCATION & TRAINING

- Nation's largest health sciences educational system
- 20 professional schools at 7 campuses
- UC trains 60% of state's medical students
- 10 Student Health & Counseling Centers

HEALTH CARE

- California's third largest health system
- Performs 50% of all transplant surgeries in California
- 40% of patients uninsured or on Medi-Cal
- \$1.4B in charity care and other benefits to the community annually



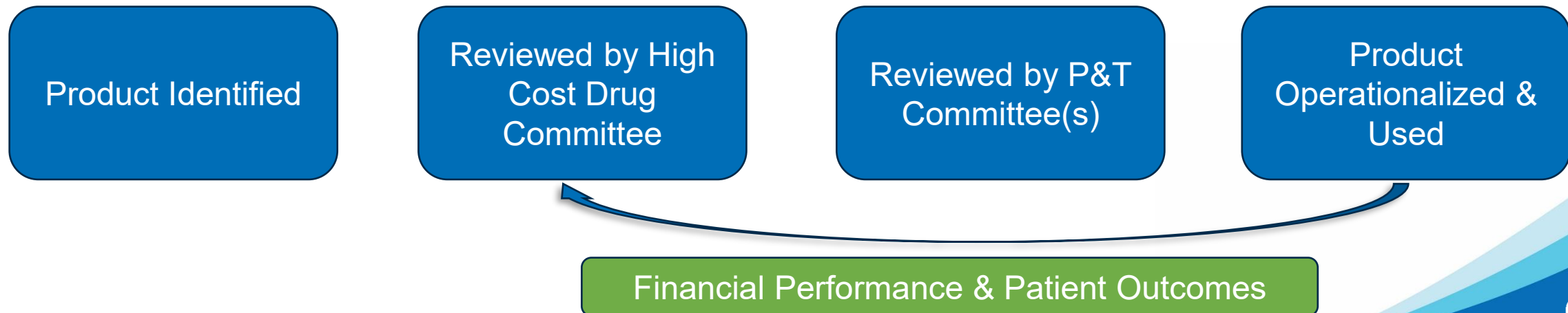
University of California San Diego



- Inpatient: 3 Facilities
 - 1,101 licensed beds
- Infusion
 - 4 Hospital Licensed Infusion Centers
 - 2 Medical Office-Based Infusion Centers
 - 1 Pharmacy Home Infusion Service
- Retail Pharmacy
 - 8 Retail Pharmacy Locations
- #1 Ranking in San Diego, 2025-2026 U.S. News & World Report "Best Hospitals"
- >42,000 annual inpatient admissions
- 1.49 million outpatient visits annually
- >\$747 million in community benefit programs and services
- First health system nation-wide to be awarded the ASHP Center of Excellence in Medication Use Safety and Pharmacy Practice certificate

High-Cost Drug Governance at UCSDH

- Multi-disciplinary structure
 - Chief Administrative Officer, Physicians, Pharmacy, Decision Support, Finance, Oncology Finance, Revenue Cycle, Payer Contracting, Others as needed Ad Hoc
- Evolving since 2018
- Current thresholds for review:
 - Singular treatment >\$15,000
 - Single patient course >\$100,000
 - Budget impact >\$500,000



Program Accomplishments & Wins

- Ensuring Pharmacy has a strong presence throughout the medication use process
 - Procurement (wholesaler & direct agreements, data agreements & information security)
 - Operational input (storage, sterile compounding, labeling, site of administration, documentation)
 - Finance (budget, charging, mark-up, program review)
 - Payer Contracting & financial assistance (authorization/letters of agreement, co-pay & patient assistance programs)
- Navigating vendor relationships

Emerging Insights

- Educate requestors on realistic timelines for onboarding
- Establish a structure for revenue cycle follow up & engage the vendor for support if needed
- Think about "out of spec" products during the onboarding / contracting process
 - Manage patient expectations related to "out of spec" product
- Evolve the process as more products across various disease states

Moving Forward

- Further evolution: High Cost Drug -> High Risk Therapies
 - Enhanced Physician involvement
- Site of Care
 - Review of facilities infrastructure
 - Centralizing authorization for high-cost therapies
- Engage all levels of pharmacy staff for development
 - Storage / inventory management
 - Sterile Compounding

Case Study 4: Strategic Partner and Internal Stakeholder Engagement

Catherine Oliver, PharmD, BCPS, DPLA
Assistant Vice President, Clinical Pharmacy Services
Ochsner Health

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About Ochsner Health

Ochsner serves patients throughout

**LOUISIANA,
EVERY STATE**

In the nation, and in more than

63 COUNTRIES

Pharmacy Enterprise

- 25 Hospital-based Infusion Centers
- 2 Ochsner Specialty Pharmacies and 20 Retail Pharmacies
- 1 Home Infusion hub and 4 non-hospital-based infusion centers

40K Team Members



4,900 Physicians & APPs

46 Owned, Managed, & Affiliated Specialty Hospitals

370 Health Centers & Urgent Care Centers

1,610,802 Patients served in 2024

4.3M Clinic Visits in 2024

90 Medical Specialties & Subspecialties

14 Consecutive Years U.S. News & World Report has recognized Ochsner as the No. 1 hospital in Louisiana

35K Active Members Enrolled in Ochsner Digital Medicine remote monitoring programs

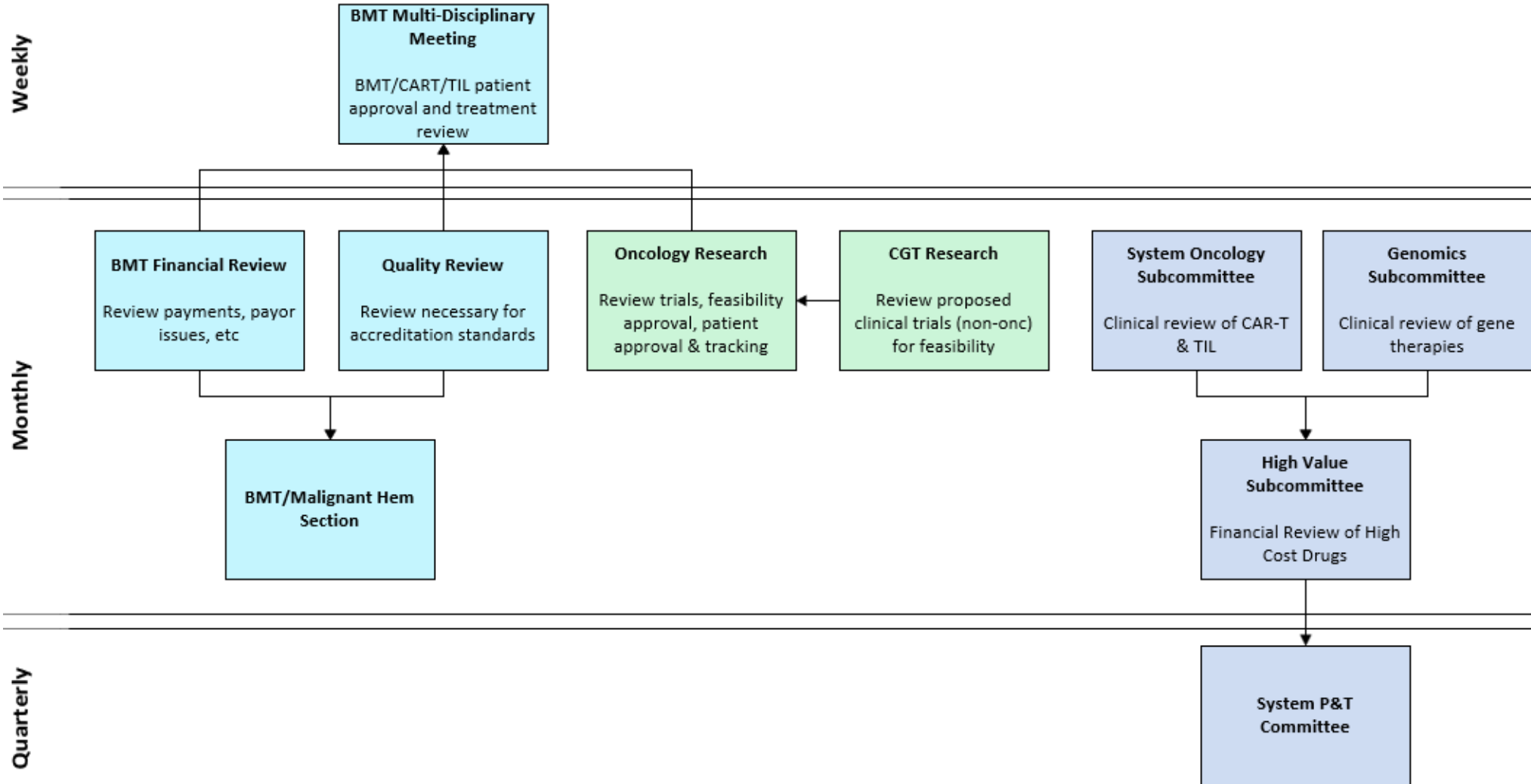
Cell and Gene Therapy at Ochsner

Patients Treated

Gene Therapies	CAR-T	TIL
Spinraza (14)* Zolgensma (2)	Breyanzi (5) Carvykti (26) Tecartus (5) Yescarta (31)	Amtagvi (1)
<i>*92 treatments total</i>		
Total = 16	Total = 66	Total = 1



Current Committee Structure

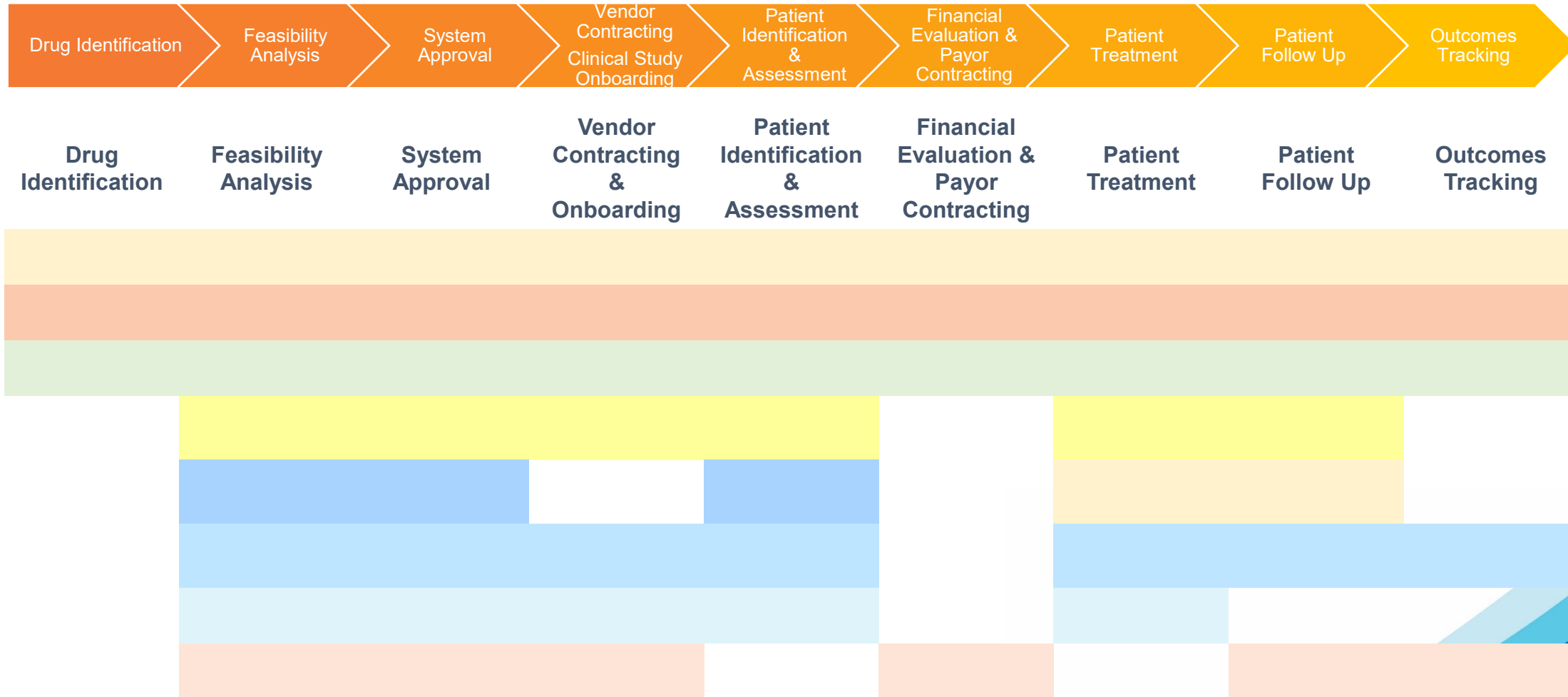


High Cost/High Value Subcommittee

- After clinical evaluation (Genomic Medicine, Oncology)
- \$20,000 per dose
- >\$100,000 per year per patient (medical and pharmacy cost)
- Anticipated system cost >\$2.5M per year

- Physician executive leadership chair, pharmacist co-chair
- Evidence-based, MUE follow-up

Process Needs as CGT Expands



Key Decision Makers

Each step of the process requires coordination between multiple stakeholders

Key Innovations and Successes

- Pharmacy is integral to all steps!
- Dedicated Program Manager role
- CGT Research Committee
 - Create pathways, grow capacity, improve feasibility beyond oncology
- Stakeholder engagement
- Current state process map

Lessons LEARNING

- COMMUNICATION, COMMUNICATION, COMMUNICATION
- More stakeholders: Quality, Accreditation, Government Relations, and Patients
- Flexibility! PIVOT!
- Still learning: “What got you here won’t get you there.”

Future Direction

- Streamline communication pathways
 - Before and during on-boarding
 - Patient selection, treatment, follow-up, outcomes
- Review and Approval Process efficiencies
- Site of Care evaluations
- Patient Selection Committee
- Cellular therapy clinical care team models

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