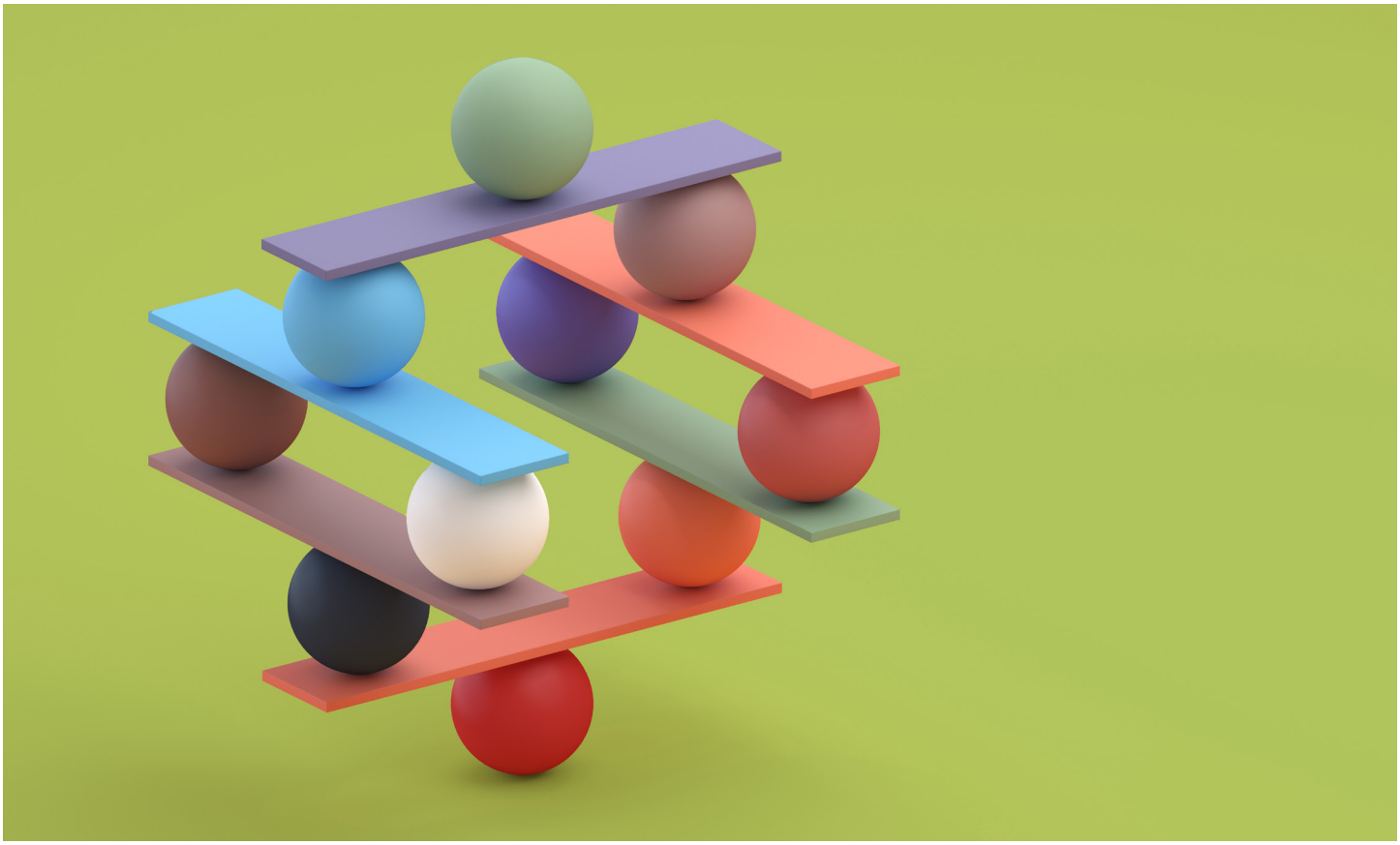
NEW DRUG DEVELOPMENT PARADIGMS INITIATIVE

TuftsMedicine
Tufts Medical Center

FoCUS

Financing and Reimbursement
of Cures in the US



DESIGN LAB BRIEFING

April 2023

CONFIDENTIAL

Tufts Medical Center Institute for Clinical Research and Health Policy Studies
Center for Biomedical System Design

April 2023 Design Lab
Agenda

Advancements and Opportunities for Precision Reimbursement
April 25 - 26, 2023
Convene at One Boston Place
201 Washington St, Floor 2, Boston

Themes
- Practical Payment Innovation
- Risk Pooling for Chronic and Durable Therapies
- Leveraging PDUFAVII for Payment Innovation

Objectives
- Investigate actionable payment innovations through a multi-stakeholder lens
- Share best practices in implementation in outcomes contracts
- Understanding policy implications of PDUFAVII

Dear FoCUS Design Lab participants,

I am writing to share an update on the FoCUS Project since our last meeting. As part of our ongoing efforts to keep our stakeholders informed, we have compiled a set of optional reading materials that showcase some of our recent achievements. These materials are intended to provide additional insight into our work and demonstrate the impact that The FoCUS Project is having in our community.

We recognize that your time is valuable, and as such, we want to emphasize that these materials are entirely optional reading. However, we believe that they will be of great interest to those who are following our progress and want to stay informed about the latest developments in our field. Should you choose to review these materials, we welcome any discussions or questions you may have.

Thank you for your continued support of the FoCUS Project. We look forward to sharing more updates with you in the future and seeing you on Tuesday, April 25th.

Sincerely,
Tsega Meshesha

Other suggested background:

Value-based performance arrangements for chronic conditions: an economic simulation of Medicaid Drug Rebate Program reforms (tandfonline.com)

Full article: Medicaid best price reforms to enable innovative payment models for cell and gene therapies (tandfonline.com)

DAY ONE	**11:30am – 7:30pm**	
11:30am – 1:00pm	REGISTRATION	
11:35am – 12:20pm	Orientation to The FoCUS Project	FoCUS Advisory Group
12:00 – 1:00pm	LUNCH	
1:00 – 1:45pm	Welcome, Introductions and Frame the Day	Mark Trusheim
1:45 – 2:30pm	Market Advancement and Opportunities, opening panel	Alice Valder Curran Scott McGoohan Gail Ryan
2:30 – 5:35pm	**Risk Pooling Strategies for Chronic and Durable Therapies: Part II Case study** Opening presentation & clarifying questions Breakout sessions Report out and Discussion	Joey Dizenhouse Michelle Harika Kelly Munger
5:35 – 6:00pm	Daily Wrap Up and Next Day Overview	Mark Trusheim
6:00 – 7:30pm	Cocktail hour	

DAY TWO	**8:00am – 1:30pm**	
8:00 – 8:45am	NETWORKING BREAKFAST	
8:45 – 12:40pm	Precision Reimbursement Adoption Monitoring (PRAM) Project	Ashley Hume
	Analysis of 340B and ASP Interactions for Precision Reimbursement & Value-based contracts	Mark Trusheim
	When could a managed entry agreement create a win-win-win situation — Exploring the window of opportunity	Marcelien Callenbach
	Payers' Perspectives on Value-based contracts best practices, barriers and challenges, areas of improvement	Chester "Bernie" Good Michael Sherman
10:50 – 11:05am	BREAK	
	Toolkit 2023 — Updates and demonstration	Karen Geary
	PAM 2023: Pipeline Updates and Expansion Opportunities	Claire White Colin Young
	PDUFA VII Policy Implications on Real World Evidence	John Glasspool
12:40 – 1:00pm	Wrap Up and Closing	Mark Trusheim
1:00 – 1:30pm	NETWORKING LUNCH (To-Go available)	

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DESIGN LAB BRIEFING, APRIL 2023

Risk Pooling Strategies for Rare Chronic and Durable Therapies (Part II: Multiple Product Pools)

Key Question: Can patient access to rare, non-oncology, chronic and durable therapies be improved by reducing the disproportionate financial burden to Self-Insured Employers (SIEs), small fully-insured payers and state Medicaid plans via an innovative multi-drug, multi-year pooling approach (A Rare Disease Pool)?

Design issues to pressure test and refine in breakout sessions:

1. Pool funding mechanisms that attract broad participation over multiple years and avoid adverse selection.
2. Connection to existing stop loss, reinsurance and gene therapy subscription products.
3. Scope of populations, therapies and ancillary costs to include in the pool.

Breakout Group structured discussions will address the following areas:

- **Breakouts 1 & 2:** Funding Mechanisms
  - **Sources:** premiums (voluntary, mandatory), government full or partial subsidies, etc.
  - **Avoiding Adverse Selection**
  - **Level of customization:** uniform coverage or tailored? Combine or separate SIE, small insurer, Medicaid?
- **Breakouts 3 & 4:** Coverage Scope and Coordination
  - **Coverage:** drug only, add medical?; all rare, above $X/pt?
  - **Coverage:** Combining durable and chronic?
  - **Relation to reinsurance, stop loss, dual eligibles**
- **Breakouts 5:** Combining durable and chronic therapies in one pool

Section 1. Background and Problem Description

High Cost Rare Disease Challenges for SIEs, Small Payers and Medicaid Plans

Novel rare disease treatments receive high prices for their effects in a small patient population. Employer-sponsored health plans are motivated to support the health and wellbeing of members – whether they are employees, retirees, or eligible family members. Similarly, Medicaid plans are mandated to provide health care to all eligible beneficiaries, which includes all FDA approved therapies that participate in the Medicaid Drug Rebate Program.

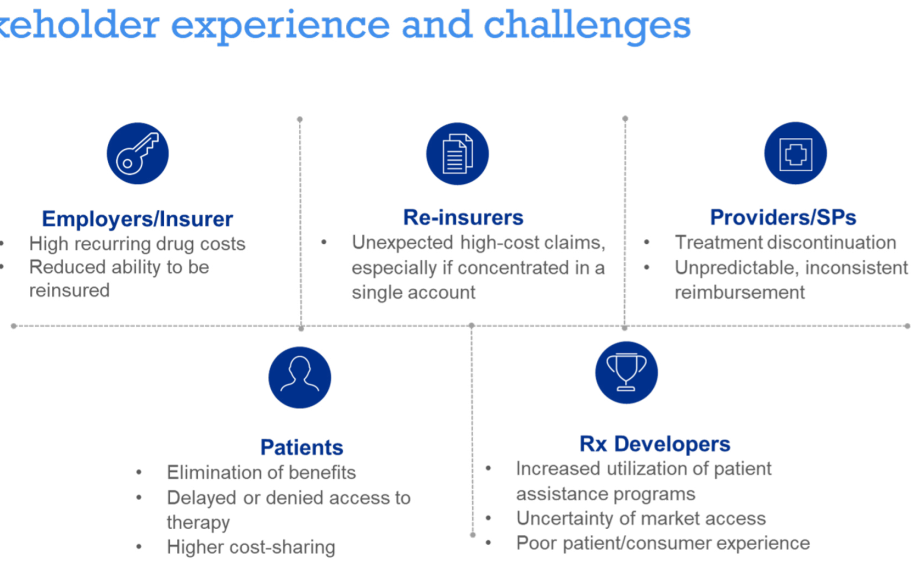
Covering non-oncology rare disease therapies presents the following challenges for smaller payers:

- The **actuarial and statistical unpredictability** of one-time gene therapy or even recurring chronic therapy expenditures pose challenges for individual employers and small insurers. While larger self-funded employer groups that provide healthcare coverage to employees and their families can absorb the cost of rare disease treatments due to their large membership pools, payers with small membership bases often struggle to pay for therapies, a problem that will worsen as more rare disease therapies come to market. VBAs do not address the financial risk from this disproportionate, statistics driven, cost burden.
- **Uncertain benefits over time** as most rare disease therapies, by their nature, can only support small clinical trials. In addition, the high unmet need of patients suffering progressive morbidity or acute mortality ethically requires rapid therapeutic access before long-term evidence is available.
- **Disproportionate cost concentration** when cases do occur in a small payer's population. As Figure 1 illustrates, with fewer members across which to spread the high cost, the relative cost impact of rare disease treatment is amplified.

Figure 1. SIEs and small plans face disproprotionate cost concentration

To reduce their financial exposure, some employers have responded by eliminating coverage for these therapies. Figure 2 lists further impacts by stakeholder type. These developments threaten to stall or even erase the remarkable progress made in treating rare diseases over the last 40 years — although patients with rare conditions may now have treatment options, they may not be able to access them because their insurance places them out of reach financially. A case for coverage of rare diseases, and even federal funding for such coverage, may provide a solution to the thorny problem of rare disease coverage.

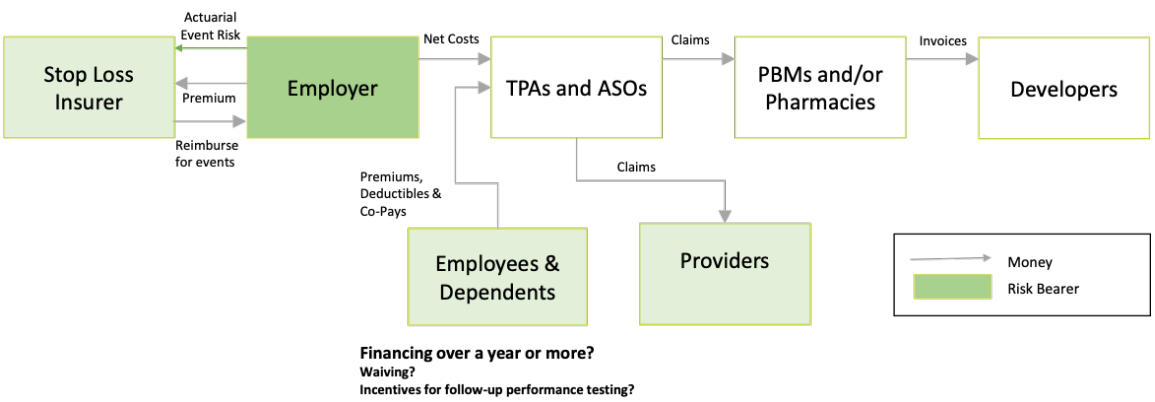
Figure 2. Impacts of Disproportionate Cost Concentration by Stakeholder



Stop loss and reinsurance provide a partial solution

Secondary insurers such as stop loss carriers, reinsurers and durable therapy financial carve-out products alter risk bearing and financial flows as illustrated in Figure 3 for employer stop loss coverage. Reinsurance offerings perform similarly for small fully insured payer plans and Medicaid plans. By pooling many employers' lives, stop loss spreads the risk of high cost healthcare events across all participating employers. Stop loss carriers charge a premium to each employer participating in the pool, which typically lasts for a single year. Each year the employer may choose whether to purchase or renew stop loss coverage from one of the X vendors.

Figure 3. Self-Insured Employer Financial Flows and Risk Bearing



Pricing insurance is complex, and the market will likely need to shift to accommodate the numerous challenges for stop loss and reinsurance carriers related to the pipeline of biomedical innovations including durable cures (cell and gene therapies) as well as high cost chronic rare disease therapies. Despite the challenges, most stop loss carriers and reinsurers are providing coverage for all FDA-approved cell and gene therapies. Today, there are also several carve out solutions currently available in the market that provide protection from the costs of cell and gene therapies for unexpected patient cases. These solutions are only for approved treatments and may need adjusting to be feasible if treatments come to market in much more prevalent conditions.

Stop loss and reinsurance products typically include "lasering" for (exclusion of) known high cost individuals to lower the SIE premium. If the SIE purchased stop loss for these individuals, the stop loss carrier would specifically include the expected covered costs for them and then add a percentage to cover the stop loss carrier's expenses and profit margin. Stop loss does not provide a pooling tool for these known high cost individuals.

However, many stop loss carriers offer a "no new laser" product that guarantees no additional lasers are added at renewal. These policies are annually renewable and most carriers are not offering "no new laser" products that are perpetual – that is, the SIE could still be at risk for lasers at the third renewal. Finally, a new stop loss carrier would also likely laser now known high cost individuals that would be identified during the underwriting and due diligence process.

Stop loss and reinsurance provide a risk pooling tool for spreading the costs of new, unexpected, high cost patients for the initial year or three. But they are not designed to provide risk pooling for continuing high costs for patients or the anticipated high cost for a known patient choosing a new therapy such as a durable gene therapy.

Findings of First Case Study: Triandra Single Product

The October 2022 Design Lab explored how to reduce the disproportionate financial burden to Self-Insured Employers (SIEs) and small payers for a single product using the 'synthetic case' based on a first to market enzyme replacement therapy (Triandra) for a hypothetical, rare, autosomal recessive genetic disease Zebra caused by a loss-of-function mutation in the ZEB gene which leads to deficient Z enzyme activity. A chronic life-long treatment for children and adults, Triandra was imagined to be a subcutaneously injected product, self-administered every two weeks at a WAC (wholesale average cost) of $500K to $1M per year (WAC).

If diagnosed in the same year as Triandra treatment initiation, stop loss and reinsurance policies assist with the costs incurred during the 1-2 year term of the stop loss or reinsurance policy. Thereafter, and possibly including the initial costs if the patient had been previously diagnosed and lasered out of (excluded from) the policy, the full cost will fall upon the SIE or small fully insured plan.

The October 2022 Design Lab participants thought the best strategy for chronic therapies like Triandra was **a 'continuum' of stop loss coverage or reinsurance followed by subscription to a national private pool for high-risk patients to cover ongoing costs**. This was thought an ideal option because stop loss ensures acute coverage for the initial expenses from the unexpected therapy event, while a national private pool would mitigate ongoing costs, and perhaps also mitigate

costs for new therapies when prior therapies had been lasered out of stop loss or reinsurance policies. This scenario was considered the best option for all stakeholders and confirmed that chronic treatments for rare diseases can benefit from innovative pooling solutions.

The October 2022 Design Lab participants preferred a multi-year financing solution which would pool the rare disease risk across many small payers, each of which would pay a premium into a Rare Disease Pool. Participants further suggested that the Rare Disease Pool(s) should be structured as one or more privately operated, voluntary participation pools each with national geographic scope to encourage price competition and choice for participating SIEs and small fully insured payers. Ideally, the participants wanted the Rare Disease Pool to coordinate its coverage timing to begin after stop loss and reinsurance to avoid double coverage and adjudication disputes. If the Rare Disease Pool centralizes contracting across payers it may increase the possibility of outcomes-based contracting through lowered per patient administration costs, increased data access and so fewer patient mobility disruptions.

A Rare Disease Pool could also benefit:

- **Patients** by ensuring ongoing coverage at their current or future employers. Eliminating copays (or other incentives) in exchange for the patient contributing to ongoing outcomes tracking or real-world evidence (RWE) for the treatment might also be included.
- **Providers and specialty pharmacy** via minimization of patient churn and fewer treatment discontinuations and restarts.
- **Developers**, by minimizing risk and may help best-price management. There may be an increased risk for rebates or discounts, but utilization may be higher.

Additional design features for a Rare Disease Pool were raised by the participants:

1. Including an optional rider for ancillary medical costs for therapy administration, patient follow-up and adverse event care.
2. Ensuring an efficient process for including new product approvals in the Rare Disease Pool, especially if the pools are multi-year.
3. Empowering the Rare Disease Pool to perform innovative contracting along with the associated coverage policy, prior authorization and utilization management activities. This could require that participating plans conform their coverage policies with the Risk Pool. This could improve patient access and patient journey management. With its larger size, the Risk Pool should also have reduced patient mobility which aids in implementing value-based contracts.
4. Guaranteeing, at a minimum, no impact on patient out-of-pocket costs.
5. Perhaps including patient indirect out-of-pocket costs such as travel to centers of excellence.

It was considered unrealistic to expect bigger payers and plans to participate in the Rare Disease Pool given their ability to self-insure due to their size.

Potential Rare Disease Pool disadvantages or fundamental challenges were also identified, including:

1. SIEs and small payers not having enough high cost members in their plan to justify the cost of selecting a pool as well as paying additional premiums and coordinating benefits.
2. Payer administrative concerns (i.e., additional resource needed); and coverage (i.e., proportion of claims paid) for payers.

3. Patient administrative burdens, access delays and access contingent upon monitoring requirements for patients.
4. Provider administrative, reporting and financial burden, especially if buy and bill or similar practices would be affected.
5. Adverse selection into the Rare Disease Pool – i.e. only those with existing high cost therapy costs subscribe, thus defeating the cost spreading across larger populations insurance design. Multi-year participation commitments (or alternatively, government mandated participation) were envisioned to mitigate the issue.
6. Lack of competition if a single national pool was mandated could lead to higher pool premiums, reduced coverage options and higher administrative costs.
7. Coverage of 'stacked' or combination therapies, especially when little clinical trial evidence or FDA label guidance exists. Applies to chronic therapies used after durable gene therapies (for example: Sprinraza or Evrysdi after Zolgensma) as well as multiple chronic therapies for a single condition.
8. Satisfying state regulatory filing needs for a multi-state pool may require a 'reinsurance treaty' and may make it difficult to combine SIEs and small fully insured plans in the same national pool.

Finally, the October 2022 Design Lab participants examining the Triandra Case Study, strongly recommended that a single product pool design lacked the scale required for actuarial stability and practical implementation. They recommended that a multi-product Rare Disease Pool be considered.

Section 2. Proposed Rare Disease Risk Pool Description

Building upon the prior case study, this case study proposes two options for a Rare Disease Risk pool that includes both durable and chronic high cost therapies for non-oncology rare conditions:

- **Option 1: a "Day 1" Rare Disease Pool** for >$100K/year pediatric or adult therapies for non-oncology orphan diseases (per FDA definition) inclusive of medical administration, patient follow-up and adverse event ancillary medical costs. **Alternatives of drug cost only or full patient carve-out could also be considered**. The pool would be funded through a PMPM premium paid by the patient's **SIE, small fully insured plan, small ACA plan, or state Medicaid plan** (Managed Medicaid or state direct). Multi-year payer commitments would be required to mitigate adverse selection. Operational roles beyond financial to be explored in this case study.
- **Option 2: Reinsurance or Stop loss followed by a "Complementary" Rare Disease Pool.** Payers would rely on their current policies to fractionally cover (up to 100%) of high cost rare disease cases. Ongoing costs are covered by the Rare Disease Pool with specific inclusion and exclusion criteria to pool funds similar to Option 1.

Option 1: "Day 1" Rare Disease Pool Primary Challenges & Proposed Design to Address

Creating a viable "Day 1" Rare Disease Pool for smaller payers faces multiple challenges. The primary ones and the teams' proposals to address them regard:

- **Participants** in the pool would be:
  - **SIEs and small commercial insurers/union plans but** could have regulatory issue from combining stop loss-like coverage and reinsurance into a single pool.
  - **Medicaid plans would help enlarge the pool size** but could complicate MBP/340B/ASP compliance (separate reporting from the same pool?) and premium setting. The CMMI pilot project may complicate or facilitate Medicaid participation.
- **Funding** for the pool will primarily come from
  - **Premiums** paid by payers on a PMPM or PMPY basis. Partial but not full experience rating is recommended for both fairness and to reduce adverse selection.
  - Optional other funding sources to consider:
    - Federal incentive to SIEs to cover rare diseases through the pool, such as by a premium subsidy (carrot) or legal/regulatory requirement (stick).
    - Federal full or partial funding contribution directly to the Rare Disease Pool.
- **Adverse selection mitigation** is envisioned through the following design elements:
  - **Requiring multi-year (3-10 year) commitment** to join the Rare Disease Pool to reduce adverse selection by increasing the likelihood that a payer will experience events and so benefit from the pool.
  - Establishing mandatory plan participation by government regulation would also mitigate the risk but is neither preferred nor likely feasible.
- **Coverage Scope** includes the populations, disorders, products, and costs included in the pool.
  - **Therapeutic areas and high cost products** proposed to include in the Risk Pool are described in Table 1.

Table 1: Coverage of possible therapeutic contexts

Therapeutic Context	Non-oncology Rare	Oncology	Not Rare High Cost (>$100K/yr)
Durable Therapy with subsequent possible high cost chronic therapy (SMA, Hemophilia, Sickle Cell)	Include	Exclude	?
Multiple concurrent chronic therapies for disorder	Include	Exclude	?
Multiple Disorder w/ multiple therapies exceed $100K threshold in total	Include	Exclude	?
Single durable therapy for disorder	Include	Exclude	?
Single chronic therapy for disorder	Include	Exclude	?

  - **A pediatric-only version** of the Risk Pool could be envisioned as well, especially if Federal funding is being considered as a significant funding source.
  - **Covered rare disease costs** could vary among drug product only, adding ancillary medical costs to full patient carve-out. The team proposes considering versions covering drug prod-

uct costs only or adding in direct ancillary medical costs including patient out-of-pocket costs for travel and similar but not lost wages.

- **Multiple therapy coverage** is also considered in Table 1. The team generally recommends coverage. When alternative low-cost therapies are available, the team also recommends that the Pool include, encourage and manage those therapies.
- **Three patient sub-populations situations to address** for each payer plan joining the pool require consideration (Table 2):
  - Newly diagnosed cases
  - Existing ongoing high cost cases
  - Existing high cost cases receiving new high cost therapy

Table 2a: Coverage of three patient situations impacts adverse selection Day One Pool Design

Patient Situation	Example	Proposal
Newly diagnosed	SMA birth	Include in Pool
Existing ongoing	Child receiving enzyme replacement therapy	Include with experience rated premium
Existing cases eligible for new therapy	Hemophilia adult wishing to receive durable gene therapy	Include for 12-24 months after new drug approval?

  - If concomitant lower-cost therapies are also required to manage the condition will the Pool cover them as well. For example, use of steroids, respirators, or similar.
- **Coverage consistency:** Preferably coverage and benefits would be aligned across all participating plans in the pool. That is, the pool would set the coverage rules so that the plan could more actively manage the therapeutic value-based contracting, utilization management and provider networks (depending on costs covered by the pool). Coordination of patient benefit designs (co-pays, deductibles, and co-insurance) might prove the most difficult with broader coordination with the plan likely needed to ensure compliance with individual and family limits beyond the covered disorder.

Option 1: Important secondary issues for "Day 1" Rare Disease Pool

Additional important but subordinate issues for "Day 1" Rare Disease Pool design to resolve were also identified. Variations of the design could range from primarily a financial offering to being engaged in therapeutic cost management to managing all costs and even becoming a full medical carve-out or specialized integrated delivery network. Table 3 suggests how the choices on these issues may align along those four models.

Table 3: Secondary Issue Choices Define "Day 1" Rare Disease Pool Variations

Challenge	Financial	Drug Mgmt Only	Drug & Medical Mgmt
Pool population changes			
• Patient mobility	Within pool		
• Adding plans (walk in problem)	Annual. Experience Rated	Annual. Experience Rated	Annual. Experience Rated
Care and Cost Mgmt			
• Claims coordination w/ other payers	☐	☐	☐
• Reimbursement	To plan, like Reinsurance	For drug to pharmacies & providers	For drug & medical costs to pharmacies & providers, etc.
• Drug contracting		☐	☐
• Provider network contracting		?	☐
• Patient eligibility & Util Mgmt		☐ drug	☐
• Care Coordination			☐
Regulatory Oversight	State Depts of Insurance	State Depts of Insurance & Federal ACA, Medicaid, Medicare, etc.	State Depts of Insurance & Federal ACA, Medicaid, Medicare, etc.
Relationship to other Stakeholders			
• Patient	Invisible	Like PBM	Like Insurer
• Providers	Invisible	Payer	Payer
• Pharmacies	Payer	Payer	Payer
• Third party administrators (TPA/ASOs)	Client	Client	Client
• Commercial fully-insured plans	Client	Client	Client
• Self-insured employers	Client	Client	Client
• Medicaid plans	Client	Client	Client
• Stop Loss and reinsurers	Competitor	Secondary dual payer	Competitor / Secondary dual payer

- **Pool population changes**
  - **Patient mobility** among participating plans should lead to greater ability to implement payment innovations. Requirements for plans to make multi-year commitments to the pool should also help. However, those individuals (or companies switching to non-participating plans) leaving the pool entirely will present the same challenges as current patient mobility.
  - **Adding new participating plans over time, the "walk in" challenge** could present adverse selection challenges per above. Assuming a perpetual plan (with existing participants able to 'roll over' to a subsequent multi-year commitment when their prior commitment ends, the pool should be able to admit new member plans on a regular basis. Fixed length pools that do not renew may find it difficult to add new plans within a few years of the pool termination date.

- **Care and cost management activities**
  - **Drug contracting** by the pool would enable it to benefit from scale and implement payment innovations such as value-based purchasing arrangements or subscription models directly with developers. If so, patient mobility issues also apply here.
  - **Provider network management** by the pool could help to ensure quality, patient access and reduce costs if the pool includes medical costs. If the pool only covers drug costs, contracting with Centers of Excellence for complex therapy administration may also prove beneficial for some therapies.
  - **Patient eligibility and utilization management** such as prior authorization responsibility to ensure appropriate patient access will prove important. That said, the financial model may still require appropriate prior utilization by the client plan as part of its coverage terms. Failure could lead to reduced or denied claim reimbursement. Also, if multiple therapies are covered for a condition the pool could create and manage step therapy policies.
  - **Care management and patient care coordination** to avoid care gaps and administrative impediments would be part of the Drug & Medical Management model but likely a minor if any part of the other two models which are further removed from direct patient care.
- **Claims adjudication & coordination** with other primary or secondary payers (dual eligibles, patient also a member of another non-participating plan, etc.) will be a needed function.
- **Regulatory oversight** of the pool would likely fall to state insurance regulators such as is done with current insurers, reinsurers and stop loss carriers. Federal rules regarding health insurance coverage will likely 'flow through' as well. The rules will likely vary depending on the status of the client plans (self-insured employers have few rules, commercial insurance some, and more for ACA, Managed Medicaid, Medicaid and Medicare Advantage plans).
- **New potential 'cracks in the system'** may be created between original payer coverage and the pool (see above consistency section).
- **Relationship to other payers and stakeholders**
  - **Patient** impact would range from invisible for the financial model to more like that of the PBM or insurer for the other models. See Coverage Consistency above for additional discussion.
  - **Providers and Pharmacies** would likely be invisible to the financial model just as they are with reinsurance. However, in the other two models, providers would likely perceive the Risk pool as a payer.
  - **Developers** may not perceive the financial model, but like providers perceive the other two models as a payer with longer time horizons (if pool size and broad participation lowers patient mobility) that could better enable value-based payment innovation.
  - **Third party administrators (TPA/ASOs), commercial fully insured plans, self-insured employers and Medicaid plans** would perceive themselves as clients of the pool. But they may be required to accept more of the pool rules regarding coverage than they do with reinsurance or stop loss products.

Option 2: "Complementary" Rare Disease Pool following Stop Loss or Reinsurance

Building upon the above, what additional changes, advantages and complications would arise from having the Rare Disease Pool begin when stop loss or reinsurance coverage ends. If the payer does not carry stop loss or reinsurance, the complementary policy would still not cover the first year of new case costs (i.e. does not become a 'First Day' policy). Using the same set of primary challenges:

- **Participants: Same as above "Day 1" design,** SIEs, small fully insured payers, state Medicaid plans
- **Funding: Remains the same as the "Day 1" design.**
- **Adverse Selection mitigation:** Similar but amplified challenge with the elimination of the 'newly diagnosed' case type. Requiring the complementary to be purchased alongside the stop loss or reinsurance (committed to at time of stop loss – so a year early) could enhance incentives for those with fewer current high cost cases to join.
  - Still require multi-year (3-10 year) commitment to join the Rare Disease Pool to reduce adverse selection by increasing the likelihood that a payer will experience events and so benefit from the pool.
  - Establish mandatory plan participation by government regulation (neither preferred nor likely feasible).
  - Only two patient sub-populations situations to address for each payer plan joining the pool:
    - Newly diagnosed cases no longer relevant due to reliance on stop loss and reinsurance policies.
    - Existing ongoing high cost cases.
    - Existing high cost cases receiving new high cost therapy.

Table 2b: Coverage of three patient situations impacts adverse selection Complementary Pool Design

Patient Situation	Example	Proposal
Newly diagnosed	SMA birth	Not included. Rely on Stop Loss & reinsurance
Existing ongoing	Child receiving enzyme replacement therapy	Include with experience rated premium
Existing cases eligible for new therapy	Hemophilia adult wishing to receive durable gene therapy	Include for 12-24 months after new drug approval?

- **Coverage consistency: largest area of complication** for multiple reasons
  - **Initial costs covered inconsistently depending on treatment timing** within the stop loss or reinsurance plan cycle. This might particularly hit durable cell therapies whose treatment courses span 6-9 months and so may often spill past stop loss or reinsurance terms.
  - **Matching coverage with primary payer and their stop loss or reinsurance** might prove complex if primary payer is more restrictive and then Rare Disease Pool absorbs costs of more generous coverage, even initial coverage. For example, primary payer covers initial chronic therapy cost and then Rare Disease Pool absorbs durable therapy cost. Conversely, if primary payer has more generous coverage than "Complementary" pool, then patients may experience reduced or denied access.
  - **"Complementary" Pool may be asked to provide "Day 1" coverage for lasered patients or treatments.** The team believes this would prove overly complicating for administration. However, the pool will need clear rules regarding its coverage for formerly lasered patients or treatments. This may also be considered in the experience rated premiums – a catch-up cost may be needed to mitigate adverse selection incentives.
- **Coverage Scope:** remains the same as the "Day 1" design Market Examples of Rare Disease Pools

Section 3. Market Examples

Multiple examples exist of new or existing pool products with at least some of the features of the Rare Disease Pools.

Market Example 1: Cell and Gene Therapy Subscriptions

Gene therapy carve-outs or gene therapy reinsurance plans have been introduced by multiple PBMs associated with large insurers. Examples include Evernorth's Embarc program, Blue Cross Blue Shield's PreserveRxSM product, and Optum's Gene Therapy Risk Protection. All three programs provide gene therapy product access to small payers for a fixed PMPM or PMPY fee.

Market Example 2: Volatility Management Program

The HealthTrust Volatility Management Program (VMP) is a supply chain support organization for groups typically >1,000 employees. The organization comprises hospital system members at HealthTrust and other self-insured employer members at CoreTrust. CoreTrust is geographically and industry sector diverse, with over 2,800 members to deliver price reductions. One of the contracted sources is pharmacy benefit management, with Optum Rx as the contracted PBM. This includes about 3 million lives, covering 330+ plan sponsors (for example: Employers, Taft-Hartley trusts, Voluntary Employees' Beneficiary Association Plans (VEBAs), Third Party Administrators (TPAs))

Cost volatility is a large and growing problem, particularly for pharmacy expenses. As new drugs for rare conditions emerge, payers anticipate an ever-increasing cost. Within CoreTrust's PBM contract was a provision that the PBM participate in developing a solution to address the problem: It was only available for its members, covering drugs that were high expense and low predictability (and with lowest risk of anti-selection).

A model was developed and launched on January 1, 2022. The Program explored different legal and operational structures, looking for the most streamlined and effective approach. The process considered various insurance schemes (flex-funding, alt-funding) and results showed that a traditional insurance approach was least risky.

Selection of drugs focused on reducing volatility without creation of selection risk. This meant including drugs that are:

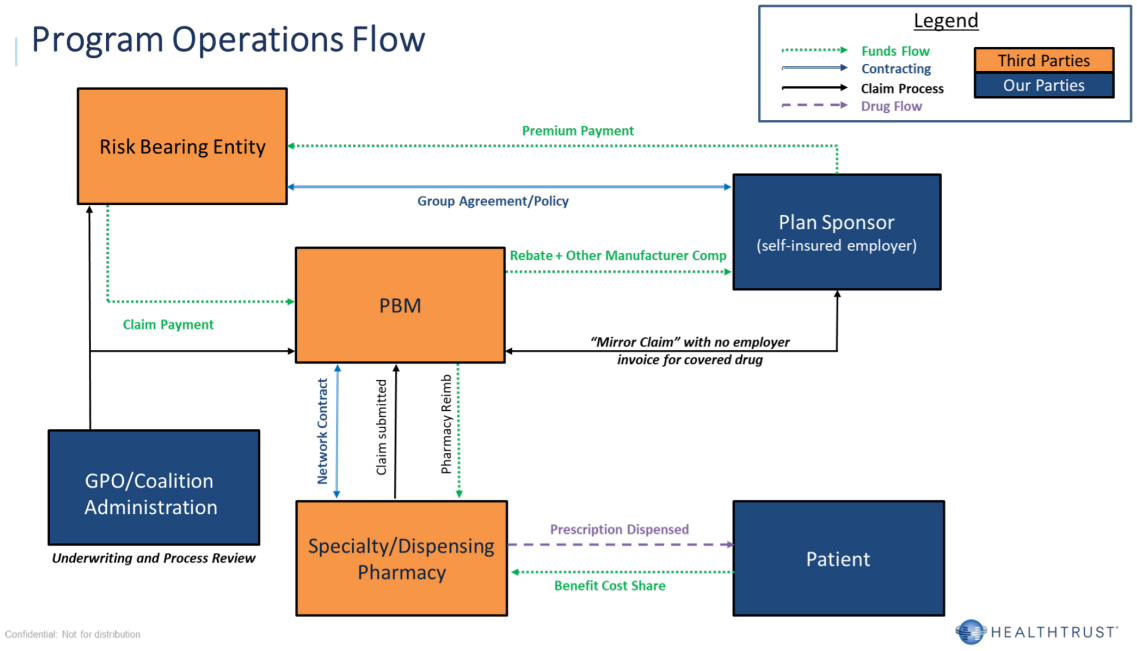
- **for rare, unpredictable diseases;**
- **on average $10K+ per prescription** (2020 data);
- **historically appropriately prescribed** (drugs which in their requirement for use is based on objective symptoms and criteria);
- **less attributable to a particular population, industry, demographic or geographic subset or possess consistent coverage practices** (exclusions, clinical criteria, site of care, presence of patient assistance.)

The program has 3-year lock-in. New members could join but those who left cannot return.

A successful program meant ensuring the process was simple. Payment would happen seamlessly without funds moving between plan years or being overly complex. For example, rather than having a payer pay a claim and then be reimbursed, the HealthTrust insurance product pays the claim directly. Addressing cost volatility meant keeping premium level modest, and not to be too "scary." This required negotiating an administrative fee approach for the PBM, with no retention of excess premium by the PBM. Premium excess or deficit would be used to establish the following year's premium.

Prior to launch, each Member Company was invited to join the program. Those joining had an addendum added to their Commitment Agreement with HealthTrust/CoreTrust. Further, each participating member was advised to work with their brokers to re-negotiate stop loss coverage This product should lower the stop loss premiums by lowering the expected submitted claims.

Figure 4: HealthTrust Volatility Management Program



After launch, the VMP participants paid a premium. All claims from plan participants for any covered NDC follow the member cost share per the plan (employer benefit) and Volatility Management Program pays remaining balance. There is no cost to the plan (employer) Claim detail noted for purposes of reporting plan performance. This ensures that the claim details are preserved (days supply, quantity dispensed, etc.). Minimum guarantees still apply, as do all other contractual benefits. A Member is completely insulated from the entire process (no change to experience). Reconciliation and other coalition support activities remain unchanged.

Market Example 3: Precision Stop Loss

Granular Insurance was launched in August 2020 as a subsidiary of Verily, an Alphabet (parent company of Google) owned company. Verily Life Sciences is a research organization devoted to

the study of life sciences including solutions that combine devices, software, medicine, and professional care to improve disease management. Granular is primarily offering Employer Stop Loss insurance to self-funded employers. They do write some reinsurance as well, primarily with captive insurance companies who are owned by self-funded employers. Granular takes a data-based approach to help self-funded employers manage risks and costs and achieve better outcomes.

Granular offers a Precision Risk approach where they segment a self-funded employer's population in to as many as 22 unique cohorts, each with different specific deductibles (healthier cohorts have lower deductibles and less healthy cohorts have higher deductibles). They require and analyze an employer's healthcare data. They are offering a multi-year rate and benefit guarantees (caps on annual premium increases). Granular Insurance has rapidly grown their employer stop loss business. They have offered very competitive pricing in the market and are offering to ease into their Precision Risk approach over a few years with their clients.

Market Example 4: Children's Special Health Care Services

Children's Special Health Care Services (CSHCS) – Michigan (CSHCS) is a program within the Michigan Department of Health and Human Services for children (under the age of 21) and some adults (with cystic fibrosis, hemophilia or sickle cell disease) with a qualifying healthcare need. They provide help to persons with chronic health problems by providing coverage, referral to specialty services, family support, community-based services to help care for the child at home, and coordination of services of many different providers. The program is not income based and all are eligible if they meet other criteria.

There is an annual fee which may be waived for Medicaid eligible individuals. Reinsurance for an HMO covering Medicaid members in Michigan may exclude coverage for CSHCS covered services. (Note: in the past individuals who qualified for CSHCS were typically excluded from reinsurance as their claims were not the risk of the HMO. It appears that this may have changed and now CSHCS helps to coordinate with the Medicaid health plan, but the HMO now may be retaining the risk.)

One issue is that out-of-state care requires special approval. There is a Children with Special Needs Fund that provides support for children with special health care needs that are not available through any other funding source. The Fund also helps purchase equipment and services to promote optimal health.

Market Example 5: High-risk patient pools

Before the ACA, **high-risk patient pools** were occasionally established to provide health insurance coverage for 'uninsurable' individuals whose high cost history led insurers to drop or deny coverage. Medicaid states also experimented with such designs to spread their costs of expected and unexpected high cost individuals. These pools often failed due to under-funding from overly optimistic cost projections leading to inadequate premiums or 'pay-ins' from the participating plans. Patient inclusion rules were sometimes poorly enforced leading to excessive patient shifting from participating payers which also led to financial distress or collapse. Finally, adverse selection death spirals were also experienced.

Section 4. Conclusion

This case study presents two options for a Rare Disease Pool, the reasons such pools might be desired, the issues to consider when designing such a pool to make it financially viable, and the numerous operational and regulatory challenges that must also be addressed.

The Design Lab participants will be asked to further refine both the issue lists and the design solutions in breakout sessions. For example, the break-outs may be asked to evaluate the two pool designs with a tool such as Table 4 below.3

Table 4: Rare Disease Pool Comparison

Criteria / Feature	"Day 1" Pool	"Complementary" Pool
Primary challenges		
1. Participants		
2. Funding		
3. Adverse Selection		
4. Coverage Scope		
5. Coverage Consistency		
Secondary issues		
1. Pool population changes		
2. Care & cost mgmt.		
3. Adjudication		
4. Regulatory oversight		
5. New 'cracks'		
Stakeholder impact		
1. Patient		
2. Provider		
3. Primary payer		
4. Channel players		
5. Developers		
6. Insurance regulation		
7. Federal programs (MDRP, 340B, etc.)		
Other?		

Section 5. Appendix

Existing pooling approaches for self-inured employer consideration are shown in Table 1a with additional pooling approaches for those who help manage SIE populations shown in Table 1b.

Table A1. Pooling Tools for Self-Insured Employers

	Pooling Tool	Features	Challenges
1	Stop Loss Insurance	Covers 100% of high -cost cases above a threshold for SIEs	Only covers first year costs; coverage may be excluded
2	Fully insured plans	Traditional insurance with pooling across insurer's covered lives	PMPM costs may be higher; benefit design less customizable
3	Purchasing Consortia/Groups	Buyer-led consortia to acquire therapies; Ex. Hospital Group Purchasing Organizations	Funding sources; Administrative burden
4	Stop Loss Carve-out; Subscription model	Access to all needed products for a fixed PMPM or PMPY. Offered by large insurers for durable cell and gene therapies.	Limited to durable therapies today; New products with little history
5	High-Risk/High Cost patient pools	SIE/Payer developed pools to segregate and share high -cost patients with fixed annual payments by each payer.	Funding levels Medicaid and private insurer attempts; Administrative burden
6	State Pools	State facilitated and run as Public Private funding partnership	Pool size; SIE participation (voluntary or mandated)
7	National Pools: Private	Mandated federal pool; Allows for 1 to a few competing pools; Funded by PMPM charge or Federal subsidy	Requires ERISA legislation; Administrative burden
8	National Pools: Government	Mandated federal pool; Funded by PMPM charge or Federal subsidy	Requires ERISA legislation; Administrative burden; May lead to single purchaser and coverage criteria

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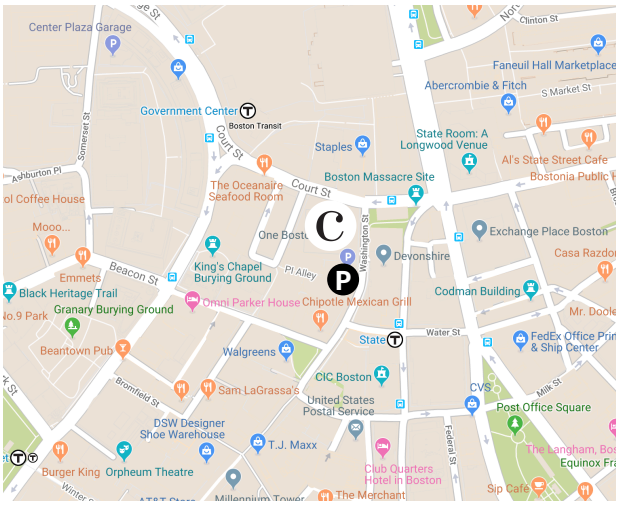

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