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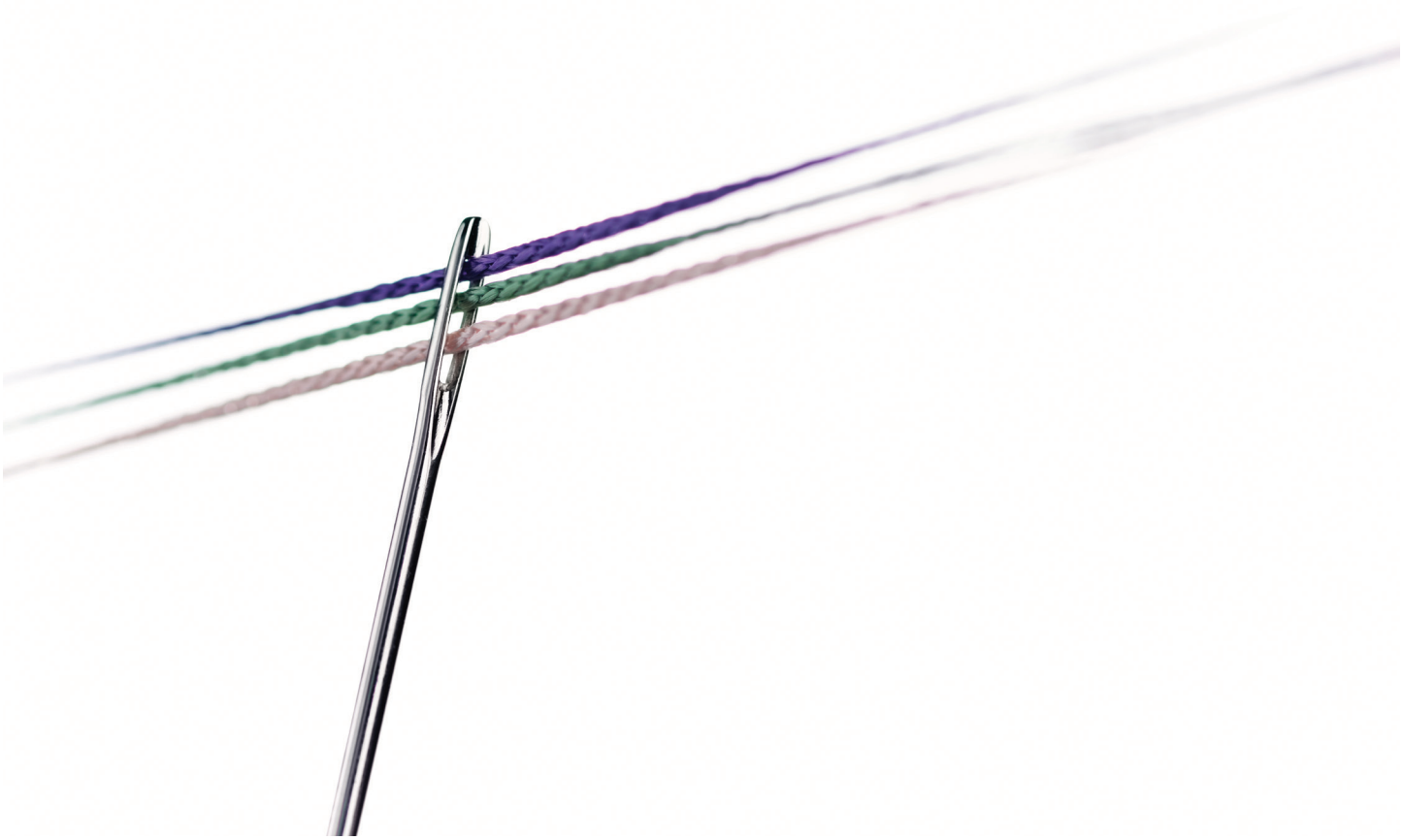

DESIGN LAB BRIEFING

October 2022

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

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DESIGN LAB BRIEFING, OCTOBER 2022

Triandra Case Study: Extending Precision Financing Pooling Tools for Chronic Rare Disease

The information contained in this briefing book is divided into three sections 1) product description 2) payer/segment mix and 3) financing challenges and proposed solutions. The product description will include development status, population effect size, market authorization, surge indication and other characteristics.

Target Area Group: Rare disease

Product: Triandra (triandramab); a synthetic case

Key Questions: Can Precision Financing pooling tools address self-insured employer (SIE) plans' financial challenges for chronic rare disease therapies?

Executive Summary of Financing Challenge(s) and Proposed Solution(s): Self-insured employer-sponsored health plans face challenges to pay for cost of unanticipated chronic rare disease treatments, particularly in instances of chronic, rare genetic diseases that may affect multiple members of a family and require ongoing treatment. Reinsurance stop-loss products as they are today may work in 'Year 1' but do not work to mitigate longer term payer actuarial risk. Using a synthetic case for rare disease, "Zebra" and a synthetic, non-curative product, "Triandra," we re-imagine risk pooling as a potential solution for the statistical unpredictability for smaller payers/purchasers that provide coverage to critical rare disease biomedical innovations.

Breakout Group structured discussions will address the following areas:

Breakouts 1 & 2: Refine the pooling solution fit by stakeholder – use Table 6 as a template.

Breakout 3: Durable therapy emphasis – Explore shared issues for durable therapy use of pooling for SIEs/small insurers with pre-existing populations such as SCD, Hemophilia and perhaps oncology.

Breakout 4: Private pooling for SIEs deep dive into design and operational considerations

- Functional scope: financing, utilization management, contracting (drug only, medical), patient navigation and care coordination
- Adverse selection and patient mobility issues
- Enrollment and attestation process burdens
- Adjudication and payment mechanisms
- Funding mechanisms which are sustainable over time

Breakout 5: Patient access perspective

- Risk of delaying access through additional process burdens
- Impact on patient out-of-pocket costs
- Enrollment and attestation process burdens
- Opportunities/challenges to connect with patient organizations and assistance funds
- Benefit of facilitating out-of-region (and out-of-network) care at centers of excellence
- Inclusion of medical management & care coordination or exclusive financial arrangement

Section 1: Product Description

1.0. Product Summary

- Product short name: Triandra
- Development status and estimated launch year: Marketed
- The major indications and general positioning or line of treatment: Indicated for hypothetical rare autosomal recessive genetic disease "Zebra," first line therapy
- Brief overview of mode of action: Triandra addresses the impact of the underlying genetic cause of Zebra by correcting enzyme deficiency.
- Therapy administration: Recommended dosage for pediatric and adult patients is 1mg/kg, self-administered once every other week as a subcutaneous injection. FDA approved label indicates dose escalation based on non-response

1.1. Disease Burden in relation to product

Rare diseases impact approximately 25 to 30 million Americans each year, across an estimated 7,000 to 10,000 rare diseases[1]. In 1983, the Orphan Drug Act (ODA) established a framework for encouraging innovation in the treatment of patients with rare disease. Decades of research has since found the ODA made a difference—since 1983, the FDA has approved more than 600 medicines for rare diseases, compared to fewer than 10 in the decade prior to ODA. Today, there are nearly 800 rare disease treatments currently in the development pipeline. Still, only about five

percent of rare diseases have an approved therapy.

Zebra is a rare, heterogenous, autosomal recessive genetic disease with multisystemic symptoms. Zebra is caused by a loss-of-function mutation in the ZEB gene which leads to deficient Z enzyme activity. Like its namesake animal's stripes, each Zebra patient is unique, though generally presenting similarly. Unlike precision treatments for genetic conditions like cystic fibrosis, use of Triandra is not tied or indicated to specific mutations or degree of severity.

People with Zebra experience widely variable symptoms. Severity ranges from fatal within the first years of life to chronic debilitation throughout adulthood. Zebra affects both males and females in equal proportions. Over 50 percent of people with Zebra are children under 18 years old. Zebra affects more Hispanic and Asian race/ethnicities than White and Black. Given that it is rare (diagnosis delay, non-specific diagnosis coding, etc), the overall incidence and prevalence of the various forms of Zebra are poorly understood but is estimated at 1:100,000 (incidence) and 1:200,000 (prevalence). Zebra diagnosis is challenging and delayed due to the numerous disorders and systemic features; adult patients experience an average diagnostic delay of 5 years. Children who present with symptoms tend to experience more severe forms of the condition. Zebra is typically diagnosed without genetic testing, except in prenatal cases. Clinical and biochemical findings – the presence of symptoms and enzyme levels – are predominant method for diagnosis. Lack of ZEB gene mutation or abnormality cannot be used to exclude a diagnosis of Zebra. Milder cases can go misdiagnosed and/or undiagnosed. Prior to diagnosis, patients with Zebra can begin their journey with primary care and family physicians, neurologists, rheumatologists, endocrinologists, and psychiatrists. Upon diagnosis, patient care is managed and ideally coordinated by endocrinologists.

Zebra is a progressive disease associated with lifelong complications. Symptoms of Zebra can appear at any age and worsen over time. Zebra can leave patients with significant disabilities – many adult patients are unable to carry out activities of daily living; however, some patients are able to maintain employment, particularly with proper control and treatment of the disease.

There are five centers of excellence treating Zebra in the US – primarily located within prominent children's hospitals and urban academic centers. These centers of excellence provide multi-disciplinary care with the assistance of nurse coordinators, as well as social services.

Table 1. Epidemiology and Demographics

Indication	Incidence (US)	Patient Backlog	Efficacy	Uncertainty of Effect	Payer Mix	Benefit Program
Zebra A hypothetical rare autosomal recessive genetic disease	1:100,000 (incidence) ~35 US births per year and 1:200,000 (prevalence) ~1,000	~1,000	Increase in survival for pediatric patients; in adults, improvements in activities of daily living, reductions in disability	Similar to other enzyme replacement therapies	60% Commercial; 20% Medicare; 20% Medicaid	Pharmacy

Triandra (triandramab) was approved by the Food and Drug Administration in 2017 as the first treatment that addresses the underlying enzyme deficiency of Zebra (Table 1). Triandra is indicated for patients with Zebra aged 3 and up. The FDA label indicates weight-based dosing, as well as dose escalation based on non-response. Annual per patient costs range from $500K to $1M (WAC). Prior to the availability of Triandra, best supportive care included physical and occupational therapy, vitamin supplements, organ transplant, and stem cell transplant. Many patients on Triandra continue some form of additive therapy (e.g. physical and occupational therapy) and nutritional supplement.

1.2. Clinical Efficacy

What is the therapeutic mechanism and treatment regimen that results in a durable effect?

Triandra is an enzyme replacement therapy that addresses the deficiency in Zebra. It is self-administered every other week through subcutaneous injection.

What is the efficacy and how is it measured?

In a prospective, single-arm trial and extension in 30 pediatric and 45 adult patients with Zebra, Triandra statistically significantly increased five-year survival. Overall, the trial found statistically significant reduction in acute events, fatigue, and improvements in quality-of-life scores. Adult patients in an open-label study showed reductions in enzyme substrates characteristic of Zebra, and reduced fatigue, pain, and disability. Real-world effectiveness was measured in a long-term effectiveness study using a multi-site and multi-national patient registry and demonstrated outcomes consistent with open label study, reduction in fatigue and improvement in quality-of-life scores.

What are significant adverse events and their frequency?

Triandra has a well-established safety profile as evidenced by five years of data. The most reported adverse reactions were injection site reactions most frequent within the first three months of treatment.

To what degree does the product address the unmet medical needs as defined by patients, clinicians, regulators, and payers?

Triandra is the first treatment targeting the enzyme deficiencies of Zebra since the disease was identified 60 years ago. For pediatric patients, Triandra represents improved mortality and greater likelihood of normal development. For adult patients, who tend to have less severe but still-debilitating symptoms, Triandra allows for less fatigue and disability – allowing patients to perform activities of daily living and participate in society in meaningful ways such as through involvement in school, employment, and family and community events. Clinicians now have an effective treatment for patients, instead of an array of symptomatic treatments that do not slow the progression of the disease. More research on Zebra and real-world evidence on Triandra and future innovations would continue to aid clinical care. For private and public payers, Triandra is a high-priced product for a relatively small population. It is difficult for any payer to anticipate whether

it will have a patient with Zebra, and, given that the condition is genetically linked, a given payer could have as a plan member more than one patient within a covered household.

1.2.1. Clinical Utility

What healthcare costs, care giver costs and social costs will the cure obviate?

Treatment will reduce overall healthcare utilization such as inpatient, outpatient, and some supportive care costs. Timely diagnosis also reduced frequent and repetitive diagnostics, specialist visits, and ineffective treatments. Most savings from treatment were indirect – patients were able to contribute to society and the economy by attending school and maintaining employment. Some caregivers were also able to seek or maintain employment and experienced improved quality of life. Mental wellness and emotional health also improved for both patients and caregivers.

1.3. Clinical Adoption and Deployment

Triandra is first line treatment for Zebra. Patients are treated primarily by an endocrinologist and managed by a multidisciplinary team. For some patients, primary care physicians also play a major role in their care. Inpatient stays are necessary for acute events related to the condition.

1.4. Coverage and Reimbursement

Triandra is covered by most health plans; however, increasingly excluded by smaller, self-insured employer (SIE) plans. Long-term coverage and access may depend upon solving for disproportionate actuarial risk. Triandra is a self-administered subcutaneous injection and classified as a pharmacy benefit.

Section 2: Payer Segmentation Mix & Challenges

Zebra patients' payer mix is 60% commercial, 20% Medicare, 20% Medicaid[1]. In some states, children with Zebra can access Title V programs that provide reimbursement. Insurer switching rates are similar to marketplace average.

1 One might expect a greater Medicaid population since half of HPP patients are estimated to be children. Reasons for the 20% Medicaid population could be due to uptake of therapy, under diagnosis, provider knowledge, etc.

Section 3: Financing Challenges and Proposed Solutions

Financial challenges may arise from the product directly or from a portfolio effect if many such products receive approval over the coming years. Teams must discuss the single product financial challenges and propose solutions and are encouraged to discuss the portfolio challenges and solutions as well. Table 2 outlines specific financial challenges by stakeholder group associated with coverage and access of Triandra for Zebra.

Table 2. Financial Challenges Per Stakeholder Group

Stakeholder (Payer Segment, Innovator, Patient, Provider)	Financial Challenges
Patients	• Elimination of covered benefits delays and/or denies patient access to therapy • Elimination of covered benefits exposes patients to full costs of therapy • High cost-sharing could also prove cost prohibitive
Self-insured employers; Self-funded employer group health plans	• High recurring drug costs for a [very] small number of patients • Predictability of the expense reduces ability to be reinsured (after Year 1) without carve-outs
Providers & specialty pharmacy	• High patient churn, treatment discontinuation, and restarts; unpredictable and/or inconsistent access • More administrative burden and infrastructure needed to navigate plan-specific denials
Stop Loss /Reinsurers	• Unexpected high-cost claims, especially if concentrated in a single account
Developers	• Risk of limited market access for product if certain payer segments cannot address financing issue • Government price reporting rules hinder manufacturers' ability to provide cost relief tailored to certain plans/insurers (payers may be unwilling to enter value-based arrangements (VBAs) for rare diseases, which require considerable infrastructure to implement) • Increased utilization of patient assistance programs and third-party foundations

3.1. Financing Challenges for an Individual Product

Paying for the high cost of rare disease innovation remains challenging. 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. The challenge of covering rare disease therapies is the actuarial and statistical unpredictability of recurring healthcare expenditures that poses challenges for the employer-provided insurance system at an individual purchaser level. 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, smaller self-funded employers with small membership base 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.

To reduce their financial exposure, some employers have responded by eliminating coverage for these therapies. 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.

3.2. Financing Solutions for an Individual Product

This case study focuses on exploring risk pooling to stabilize health plan coverage of high-cost chronic rare disease therapies. The solution we envision broadly involves distributing the financial risks, and costs of rare diseases across a larger pool of patients. This can be achieved through an add-on insurance product for rare diseases. This add-on insurance product can be designed by reinsurers/third party administrators/health insurers/pharmacy benefit managers/manufacturers and can have a per member per month premium to participate.

Pooling is an approach to mitigate the uncertainty around paying for high investment therapies and address actuarial risk management. Risk pooling strategies differ and have been successful in durable cell and gene therapies. Risk pooling allow flexibility for payment innovation and fit the needs of various stakeholders. These strategies are an approach to reduce variability by increasing the effective number of covered lives across which risk is spread. Risk pools may or may not include total patient medical and drug costs but contribute to patient access and health outcomes. Implementing the appropriate solution can be challenging but understanding the constraints and challenges can encourage innovation and serve as a method for self-insured employers and health plans to provide life changing chronic therapies in an equitable manner. Risk pools are a program or coverage specific insurance product in which a premium is set and paid for coverage of a defined treatment for a group of individuals, thereby creating cost predictability. Issues to explore are risk pool size and sufficiency as well as corridors to implementation.

Existing pooling approaches for self-inured employer consideration are shown in Table 3a with additional pooling approaches for those who help manage SIE populations shown in Table 3b.

This case emphasizes SIE pooling tools:

- Stop Loss Carve-out (using a subscription model) – A subscription model helps payers manage total budgetary cost of a medicine and to some extent actuarial uncertainty for the payer around how many patients might be taking a particular therapy by establishing a fixed fee for a given year for either a target level or unlimited drug supply. A multi-year subscription can also mitigate the actuarial risk of a surge from patient backlog. It can be structured to help align public health, payer and manufacturer incentives to support increased patient access to medicines.[ii]
- High Risk/Cost Patient Pools – Create a specific inclusion and exclusion criteria to pool funds. These patients would have greater health needs and more likely to purchase higher premiums. Traditional payment for Zebra and other rare diseases, no need for strict medical management and prior authorization if therapy is a good fit and effective for the patient.

Suggested approaches to pooling include:

- State Pools – State agencies may form a risk pool with a carve-out that may be used to pay for patient therapies. State pool could be facilitated and run as a public-private funding partnership to focus solely on the incidence and prevalence of Zebra within a state or region. Historical experiments raise caution. Prior to the Affordable Care Act, some states established high risk pools to aid patients with high-cost pre-existing conditions who were either priced out of insurance markets, refused coverage, denied employment due to insurance cost concerns, or some combination of these and other factors. The experience of these risk pools was generally poor due to inadequate funding for the costs of the patients included.[iii]
- National Pools: Private – Privately funded programs available for all rare diseases or only Zebra. Collected on a national basis to increase the pool size and funds. Possibly administered and management by a nonprofit or existing foundation.
- National Pools: Government – Federally administered and managed risk pool which combines the risk-bearing of reinsurers with the therapy contracting capabilities of pharmacy benefit managers, the provider network building and medical management capabilities of insurers, and perhaps a specialty pharmacy distribution capability. A national pool covers more lives and addresses the issue of population prevalence for rare diseases.

Table 3a. 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

Table 3b. Pooling Tools for Those Who Serve Self-Insured Employers

	Pooling Tool	Features	Challenges
1	Reinsurance	Covers fractional (up to 100%) of high-cost cases above an attachment point for insurers	Only covers first year costs (uncertainty after year 1)
2	High- Risk/Cost patient pools	Includes rare disease patients with full therapy funding for a fixed PMPY rate for each SIE/small insurer	Maintaining sustainable rates and broad SIE/small insurer participation
3	Orphan Reinsurer and Benefit Manager	Combines the risk-bearing of reinsurers with the therapy contracting capabilities of pharmacy benefit managers, the provider network building and medical management capabilities of insurers, and perhaps a specialty pharmacy distribution capability.	Defining scope of services and patient pool
4	National Pools	See Table 3a line 7 and 8	See Table 3a line 7 and 8

A further design decision regards the scope of the products, patients and costs to include in the pool. Figure 1 and Table 4 describe a range of options from single product pools to including all medicines for rare diseases with multiple potential groupings between these extremes. Including ancillary medical costs or just the therapeutic costs is another scoping decision – this case study begins with a 'therapeutic cost only' scope but breakout groups are encouraged to consider the desirability of including medical costs as well.

Figure 1 Pooling Product Scope

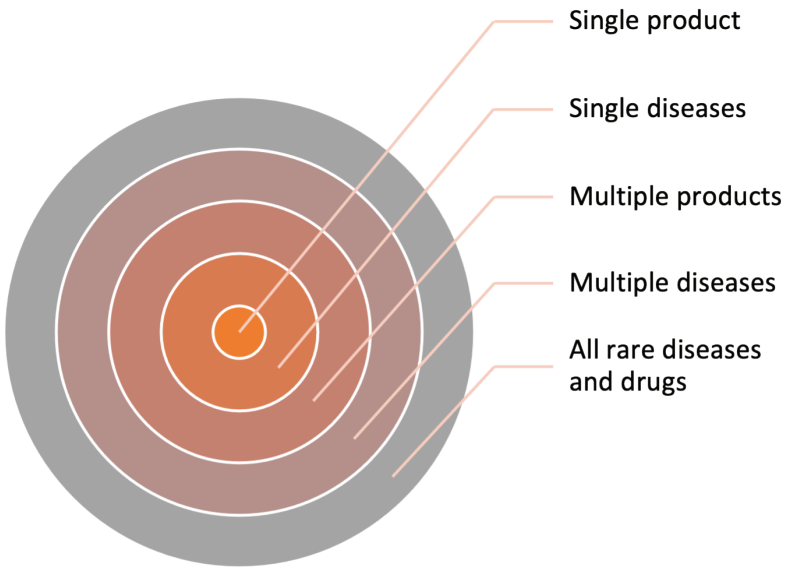

Table 4. Pooling Approach and Its Features

	Pooling approach	Features
1	Single product	Only Triandra for Zebra patients
2	Single disease	Only Zebra patients, includes Triandra and other supportive therapies and care
3	Multiple products	Subset of orphan products that meet specified criteria (e.g. enzyme replacement therapies for genetic disorders, drugs with single orphan indication, drugs exceeding price threshold)
4	Multiple diseases	Covers all therapies and services for subset of rare diseases (e.g. prevalence, genetic conditions, etc.)
5	All rare diseases/drugs	Covers all therapies and services for all rare diseases

3.3. Implementation Issues

Table 3a shows feasible pooling strategies self-insured employers can use for chronic therapies treating rare disease. This case emphasizes SIE pooling tools and Table 5 illustrates implementation considerations by five risk pool solutions for SIE.

Table 5. Implementation Considerations by Risk Pool Solution

Implementation consideration	Stop Loss Carve-out; Subscription model	High Risk/Cost patient pools	State Pools	National Pools: Private	National Pools: Government
Challenges of Innovative Financing	Limited to durable therapies today; New products with little history	Administrative burden of ongoing pharmacy and medical claims	Administrative burden; coordination with SIE/payers vs. program at individual level	Requires ERISA legislation; Administrative burden	Requires ERISA legislation; Administrative burden; May lead to single purchaser and coverage criteria
Underwriting and required number of covered lives	New chronic service – require higher premiums or deductibles.	Strictly define eligibility for participation	Mandate participation within state	Mandate participation within the national payer	Establish mandatory participation guidelines, enabling some employers to qualify to opt out.
Funding contributions	New chronic service – require higher premiums or deductibles.	Strictly define eligibility for participation	Mandate participation within state	Mandate participation within the national payer	Establish mandatory participation guidelines, enabling some employers to qualify to opt out.

/responsibility	SIE premiums	SIE premiums + premiums from other payers	SIE premiums + Medicaid + fully insured premiums	SIE premiums; other commercial payer premiums	Taxes; SIE premiums; other commercial payers
Products included/Scope of the pool	Triandra + ?	Triandra + ?	Triandra + ?	Triandra + ?	Triandra + ?
Covered costs	Therapy only	Therapy + Medical	Therapy only	Therapy only	Therapy + Medical
Inclusion criteria	No broader than FDA label criteria for all included products	No broader than FDA label criteria for all included products	No broader than FDA label criteria for all included products	No broader than FDA label criteria for all included products	No broader than FDA label criteria for all included products
Exclusion criteria	outside the inclusion criteria	outside the inclusion criteria	outside the inclusion criteria	outside the inclusion criteria	outside the inclusion criteria

In Table 5, we emphasize five risk pooling solutions for Zebra and Triandra and explore various issues that could impede adoption:

1. Challenge of innovative financing – Risk pools have predominantly been used for durable therapies or limited medical episodes. Zebra will require life-long administration of Triandra and given clinical effectiveness results, will increase the prevalence of the disease. Also, employers may not contract for coverage until stop loss coverage will not pay a submitted claim for Triandra.
2. Underwriting and required number of lives – Sufficient premium to cover claims is a function of the Per Member Per Month (PMPM) premium and total number of lives. Employers may determine to accept the risk of covering a beneficiary with Zebra if the premium is too high. However, setting a low premium does not guarantee adequate number of enrolled lives.
3. Coverage decisions – The ongoing annual costs of Triandra has inspired discussions of risk pooling. Limiting coverage to "product only" minimizes exposure of the risk pool. However, inclusion of provider services required for the comprehensive treatment of Zebra (lab tests, endocrinologist evaluations, etc.) provide for better overall management and outcomes. The mainstay of Zebra treatment before Triandra has been physical and occupational therapy, vita-min supplements, organ transplants and stem cell transplant. The option to cover by diagnosis will define different premium requirements than will coverage based on required patient use of Triandra.
4. Contract responsibilities of Risk Pool – Specialty pharmacies have drug discounts contract-ed with the PBMs of the SIE and these contracts may be used for the risk pool. Contracts may also be established directly between the risk pool and the PBM or the risk pool and the specialty pharmacy. Any additional discounts, such as rebates, may be administered by the TPA/ASO or PBM. Another option would be for any rebates to be received by the risk pool and used to lower premium requirements. Introduction of value-based contracts should consider scope of outcome data needed and its availability from the services covered by the risk pool.

Key criteria for determining the most appropriate risk pool solution are payer mix and the cost of Triandra. As previously relayed, Zebra patients' payer mix is 60% commercial, 20% Medicare and 20% Medicaid. Annual per patient costs range from $500K to $1M (WAC).

3.4 Solution Evaluation

Table 6 is illustrative of how stakeholders may assess the value of risk pooling tools in addressing their financial issues. Breakout groups are encouraged to assess which financing issues for which parties do the options address and not address.

Table 6. Assessment of pooling tools by stakeholder

Stakeholder / Risk or Requirement	Stop loss/ subscription	High risk/ cost patient pool	State Pool	National Pool (Private)	National Pool (Government)
Self-insured employers; Self-funded employer group health plans					
High recurring drug costs for a very small number of patients	x	x	x	x	x
Predictability of the expense.	x	x	x	x	x
SIE defined benefit scope/features	x	Maybe	Maybe	x	Maybe
Patients					
Improved access to therapies and care	x	x	x	x	x
No copays/ deductibles for patients	x	Maybe	x	x	x
Seamless transitions between employer sponsored plans	x	x	x	Maybe	x
Providers & specialty pharmacy					
Minimize patient churn, treatment discontinuation, restarts due to access issues	x	x	x	Maybe	x
Realize greater administrative efficiency	Maybe	Maybe	x	x	x
Stop Loss / Reinsurers					
Manage underwriting risk of single SIE account	x	x	x	x	x
Developers					
Minimize risk of limited product access if certain payer segments cannot address financing issue	x	x	x	x	x
Government price reporting rules hinder developers' ability to provide cost relief specifically for certain plans/insurers	x	x	Maybe	x	Maybe
Lessen need for developer funding of patient assistance programs	x	x	x	x	x

Regardless of the pooling tool utilized, there is value as well as risk for each stakeholder.

Employer:

- Financial benefits: lower health care spend, predictability of drug expense could help design better benefits/plans, some financial benefit by not having to renegotiate reinsurance catastrophic coverage every year or post a stop loss trigger. Higher likelihood of reinsurance renewal rates.

- Risk: Increased health premiums per member. If employers do not have a rare disease patient, risk pool premiums could be seen as waste

Patient:

- Financial benefit: Pool benefit designs could reduce/limit out of pocket costs for rare disease patients
- Risk: Potential restrictions on providers; loss of coverage if innovation of risk pool is not successful.

Developers:

- Financial benefit: risk pools ensure that patients have continued access to the therapies they need and achieve expected/promised health outcomes, increase business predictability, allows better utilization of patient assistance program funds.
- Risk: Managing the regulatory requirements for pricing and price reporting, anti-kickback statutes, etc.

Providers:

- Financial benefit: minimizes risk of incorrect or prolonged coverage determination or coverage loss.
- Risk: Adequate reimbursement and coordination of billing between insurance risk pool and health plan or TPA; clarification of services to be billed under risk pool, if any.

3.5 Risk Pool Insurers

Stop Loss Insurance Financial Flows and Risk Bearing

Risk pool insurers alter the flow of money based on how services are implemented. Figure 2 shows the flow of funds in the healthcare system.

Figure 2. Healthcare System Financial Flows and Risk Bearing

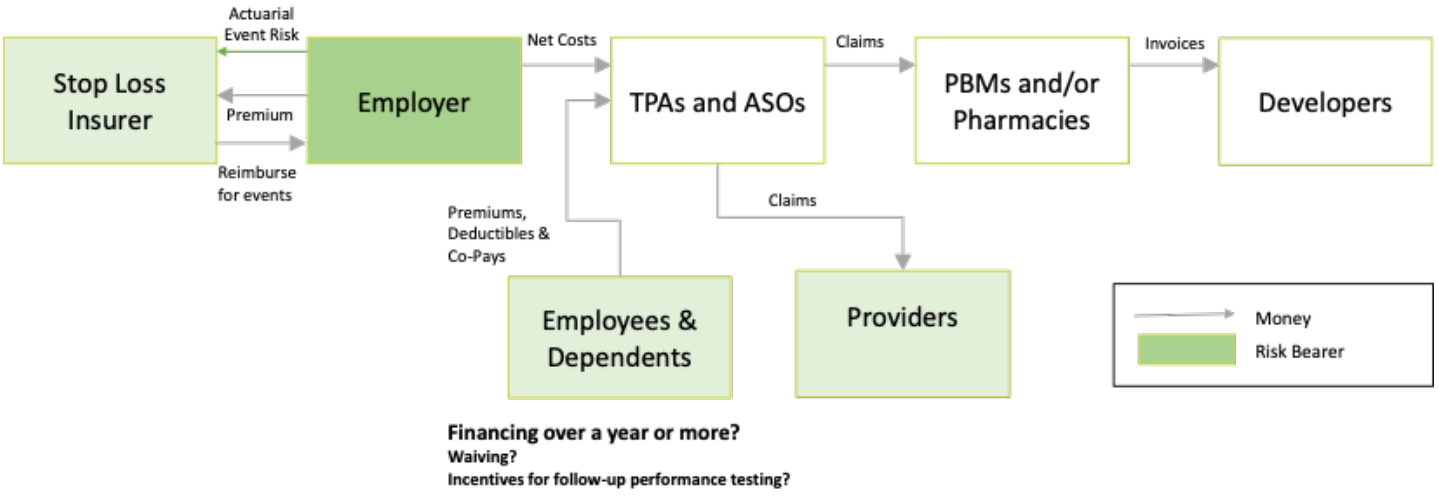

Table 7 provides a limited illustration of potential options to implement a risk pool to the existing healthcare financial systems.

Table 7. Implementation of Financial Arrangements in a Risk Pool Solution

Implementation Element	Option A	Option B
Responsibility for premium payment	Employer	Stop Loss Carrier
Provider Claims covered	Pharmacy only	Pharmacy & Medical claims
Patient Cost Sharing	Determined by SIE	Defined by Pooling Risk Insurer
Provider / Pharmacy contracts	Use contracts of TPA / ASO	Providers / pharmacies contract with pooling risk insurer
Developer rebates / discounts	No impact on terms that exist with TPA / ASO arrangement	Developer establishes with risk pool insurer responsible for paying claims.

4. Conclusion

While employers provide health benefits to maintain the health and wellness of their workforce and families, small self-insured employers at times struggle to cover the cost of recurring rare disease therapies. Financial strains within the current payment system could lead to eliminating coverage of some therapies all together. New Precision Financing pool tools provide a possible path to solving the multi-year actuarial and statistical unpredictability for small healthcare purchasers. This case study used a hypothetical condition (Zebra) and therapy (Triandra) to illustrate how different pools could be constructed and each model's implementation considerations and mitigation strategies. Ultimately, stakeholders must strive to align on the principles of protecting patients and their families, as well as designing a sustainable funding pathway.

i National Institutes of Health, NIH Study Suggests People with Rare Diseases Face Significantly Higher Health Care Costs (Oct. 22, 2021).
ii FoCUS Project, Toolkit Home
iii FoCUS Project, Toolkit Home