

Payer Access for Orphan Diseases

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Agenda

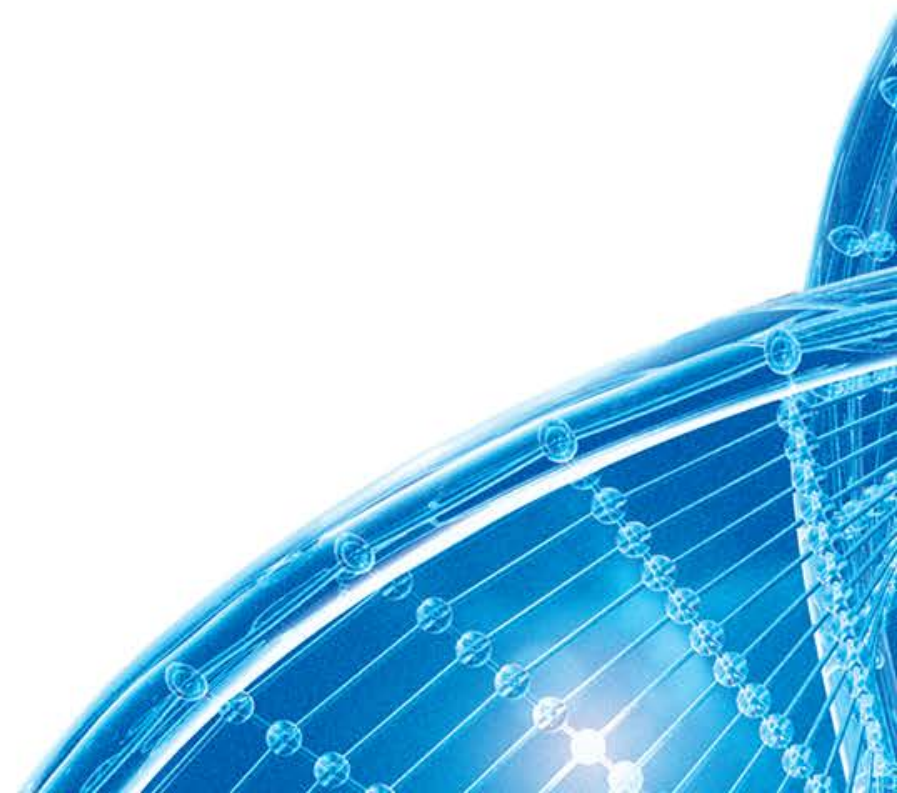
Evolution of U.S orphan disease marketplace

Growth of specialty market

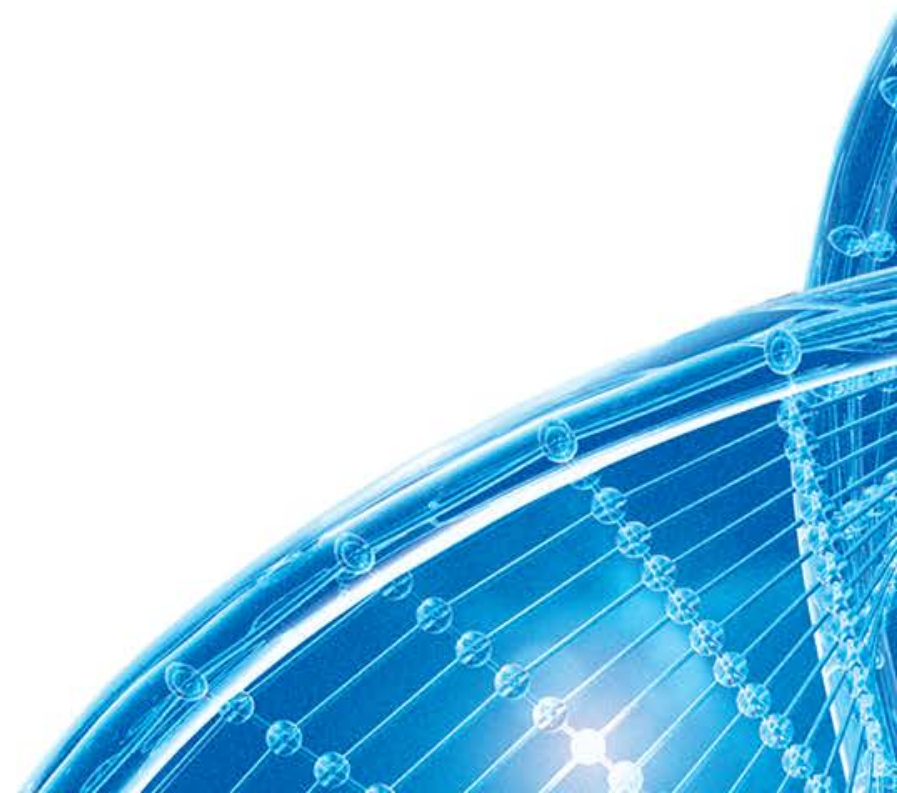
Precision medicines

Access challenges and successes

Outstanding questions facing the industry



Current Trends



Utilization Management: Differences with Orphan Drugs



Preferred
Products/Rebates

Prior Authorization
(PA)
using clinical
trial exclusion
criteria

Step Therapies

Coverage
Restrictions/Formulary
Exclusions

Evolving National Trends Shape Policy Environment



Continued Pressure on Drug Pricing

- ▲ State Price transparency bills & Task Force creation
- ▲ Admin focus on importation & gov't negotiation
- ▲ Price increase scrutiny; rationale for list price decisions

Insurance Benefit Designs

- ▲ Potential for high deductible plans/OOP costs/excluding items from MOOP
- ▲ Narrower networks

Access

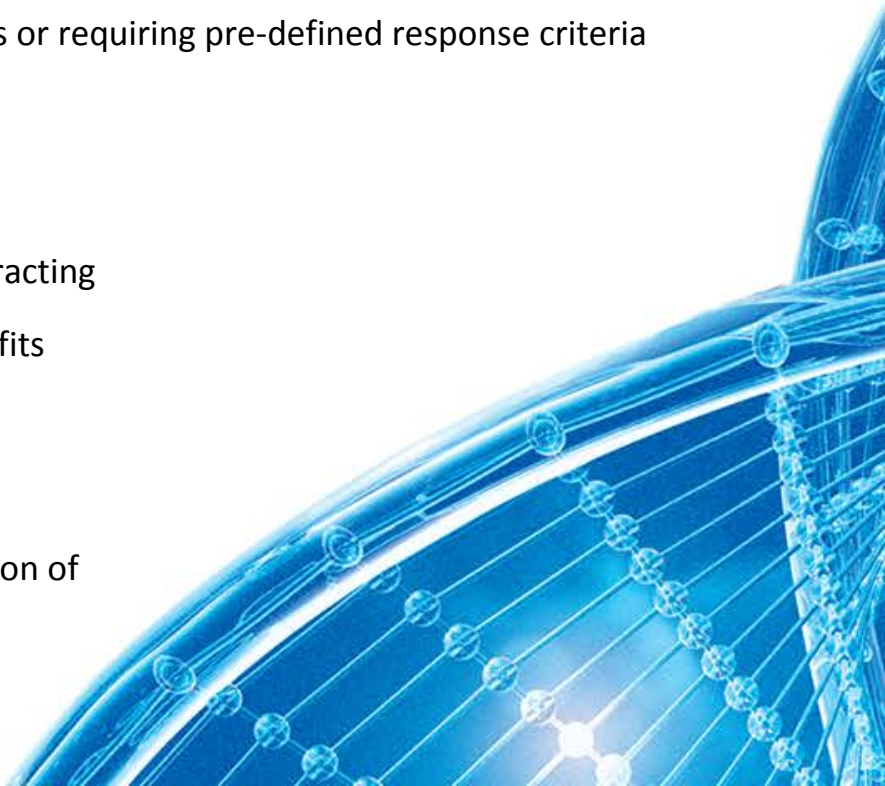
- ▲ Limiting access to specific sub-populations or requiring pre-defined response criteria
- ▲ Shift of risk in provider and payer models
- ▲ Patient cost-sharing increases

Emphasis on Value

- ▲ More Experiments with value-based contracting
- ▲ Use of RWE to substantiate product benefits
- ▲ Evolving approaches to evaluate value

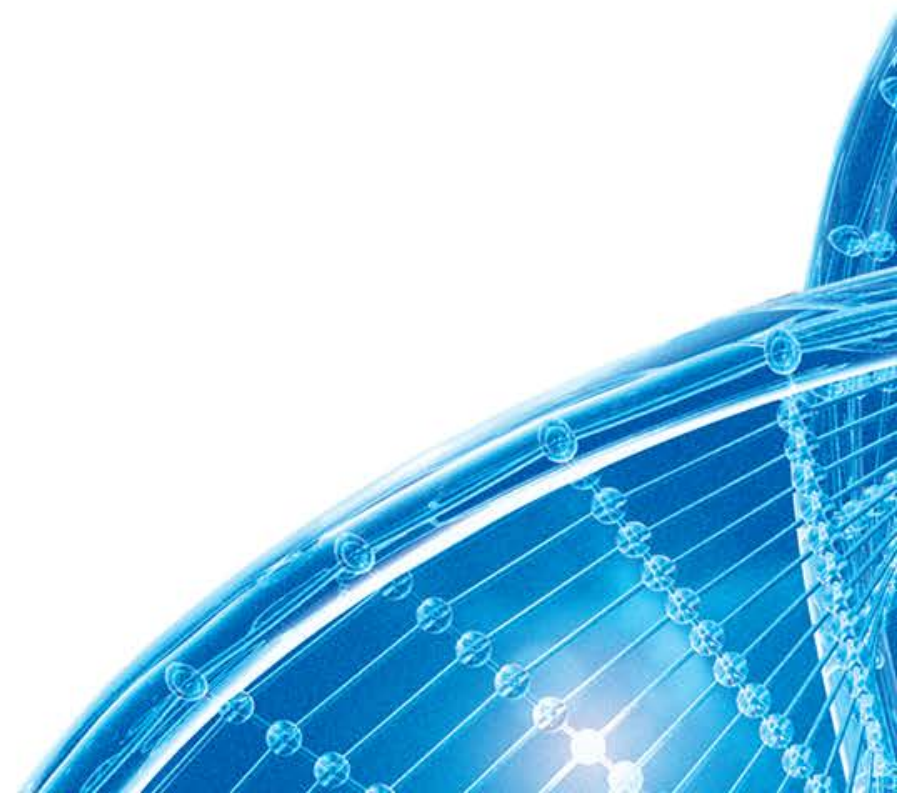
Precision Medicine

- ▲ Increased predictability for payers
- ▲ Complexity of lab testing and differentiation of products based upon mutations

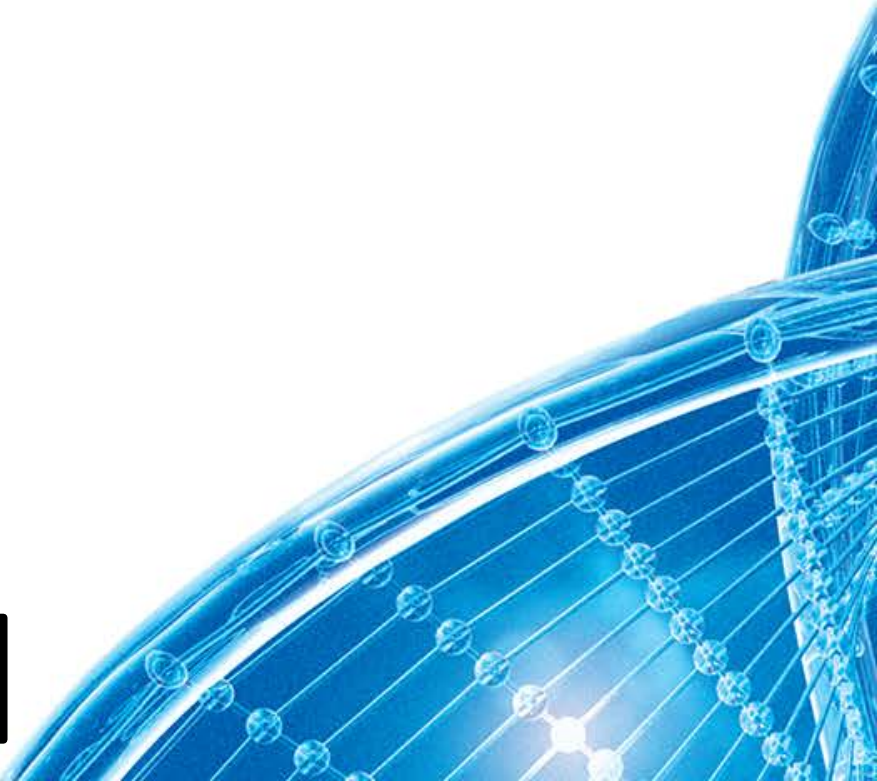
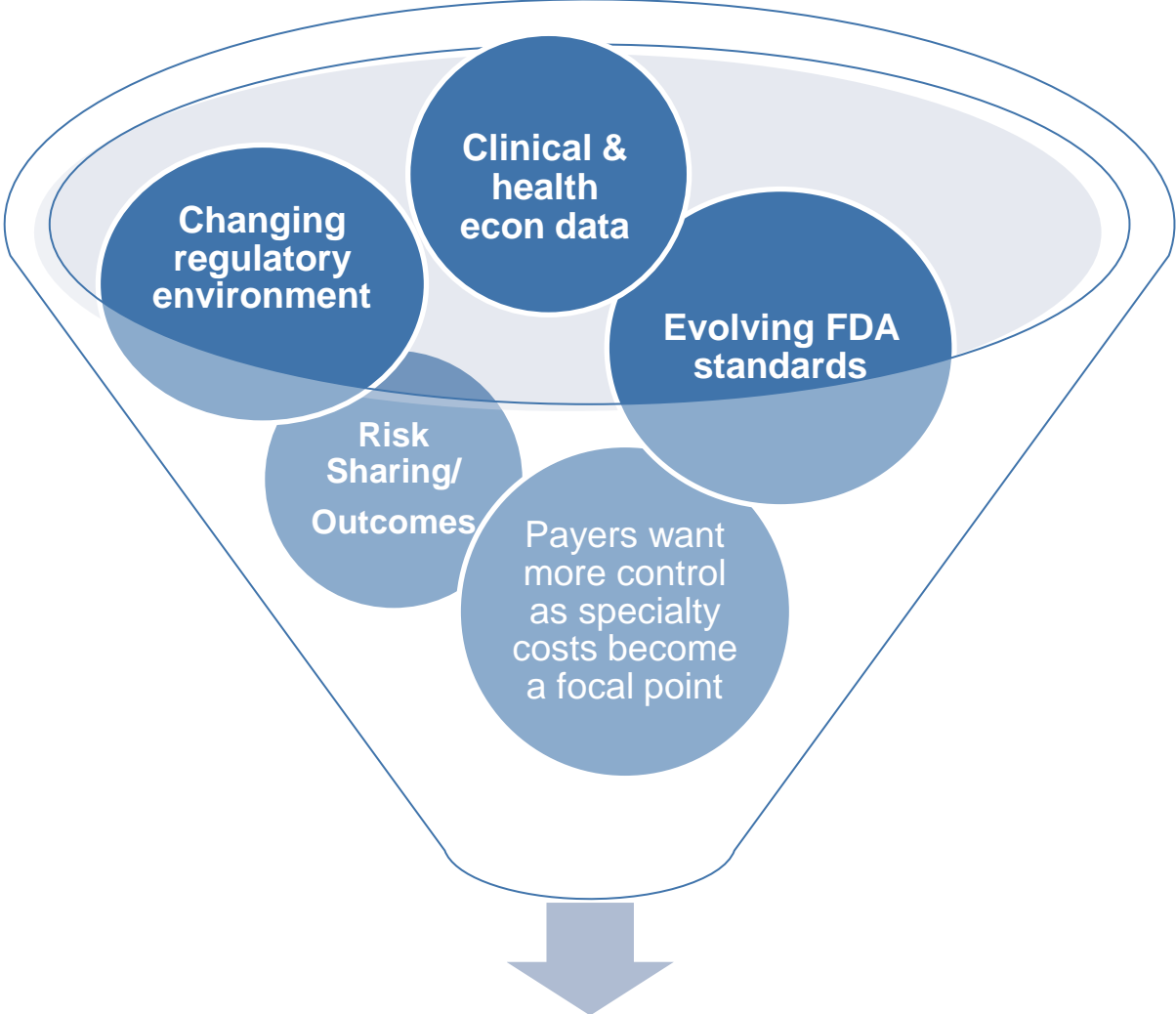


Future

sPCMA Business
Forum **2017**



What Does the Future Hold?



Questions?



THANK YOU

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