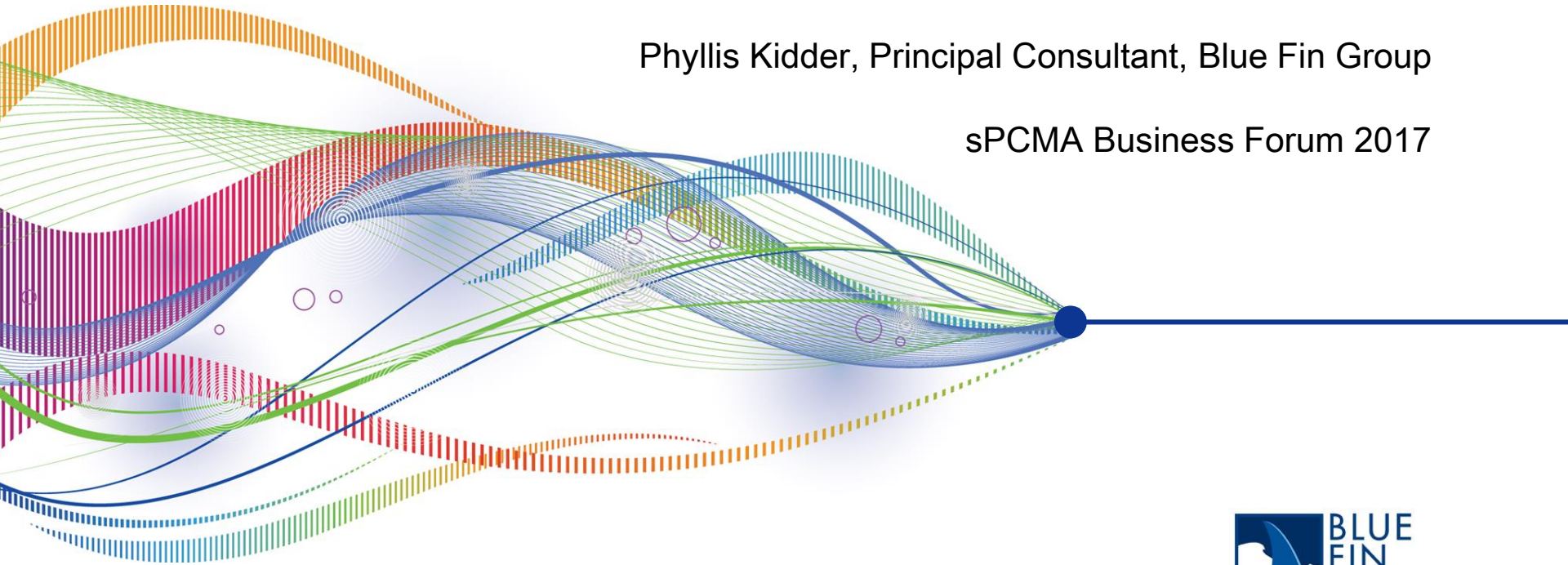


Orphan and Rare Disease Products Exclusive Pharmacy Networks

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sPCMA Business Forum 2017





Multiple Perspectives

Pharmacies

- \$75B opportunity by 2020
- How to win access
- Nuances along the way
- When and how to engage
- Importance of empathy and passion

Manufacturers

- It's your whole company
- It's the fate of your new company
- Detailed design matters
- Success or failure of a product
- Facing headwinds and telling many customers NO



Topics for Discussion

- Size of the orphan drug (ORD) market
- Network development
- Constituent frustrations
- Setting a multi-year vision – indication & patient expansion

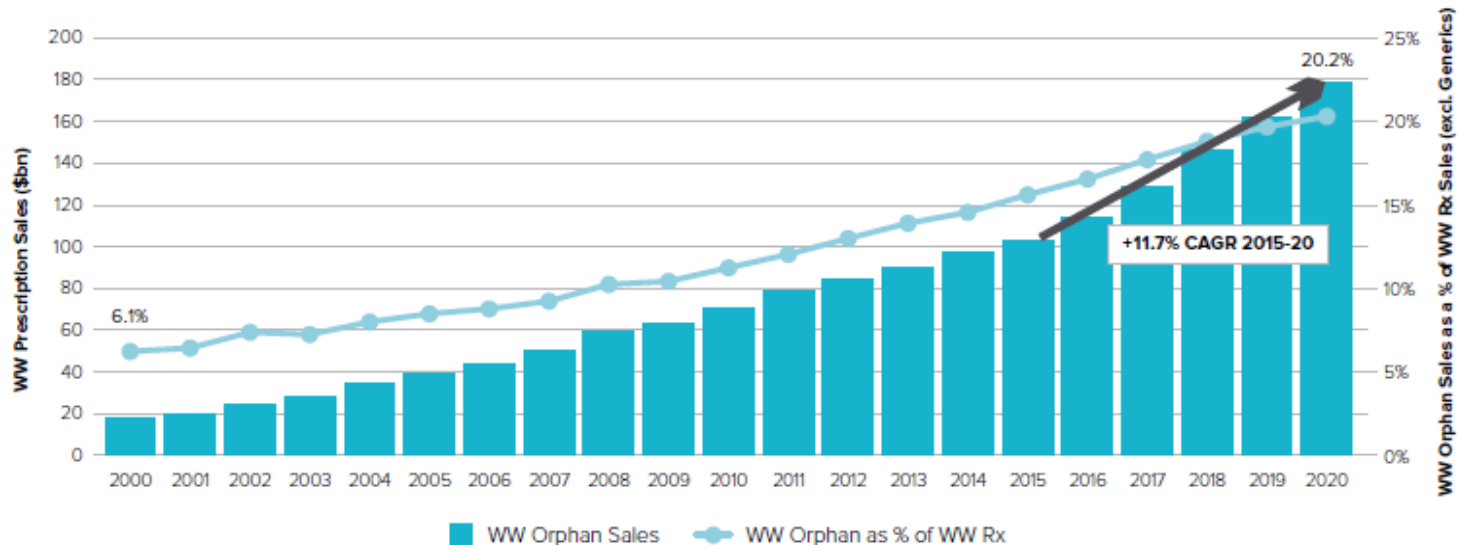


Worldwide Orphan and Rare Disease Market

- Orphan drugs will be 20.2% of worldwide Rx sales by 2020*
- Total of \$178B by 2020 (CAGR 2015 to 2020 +11.7%)
- Estimated that \$75-78 million will be the orphan drug market in the US

Worldwide Orphan Drug Sales & Share of Prescription Drug Market (2000-2020)

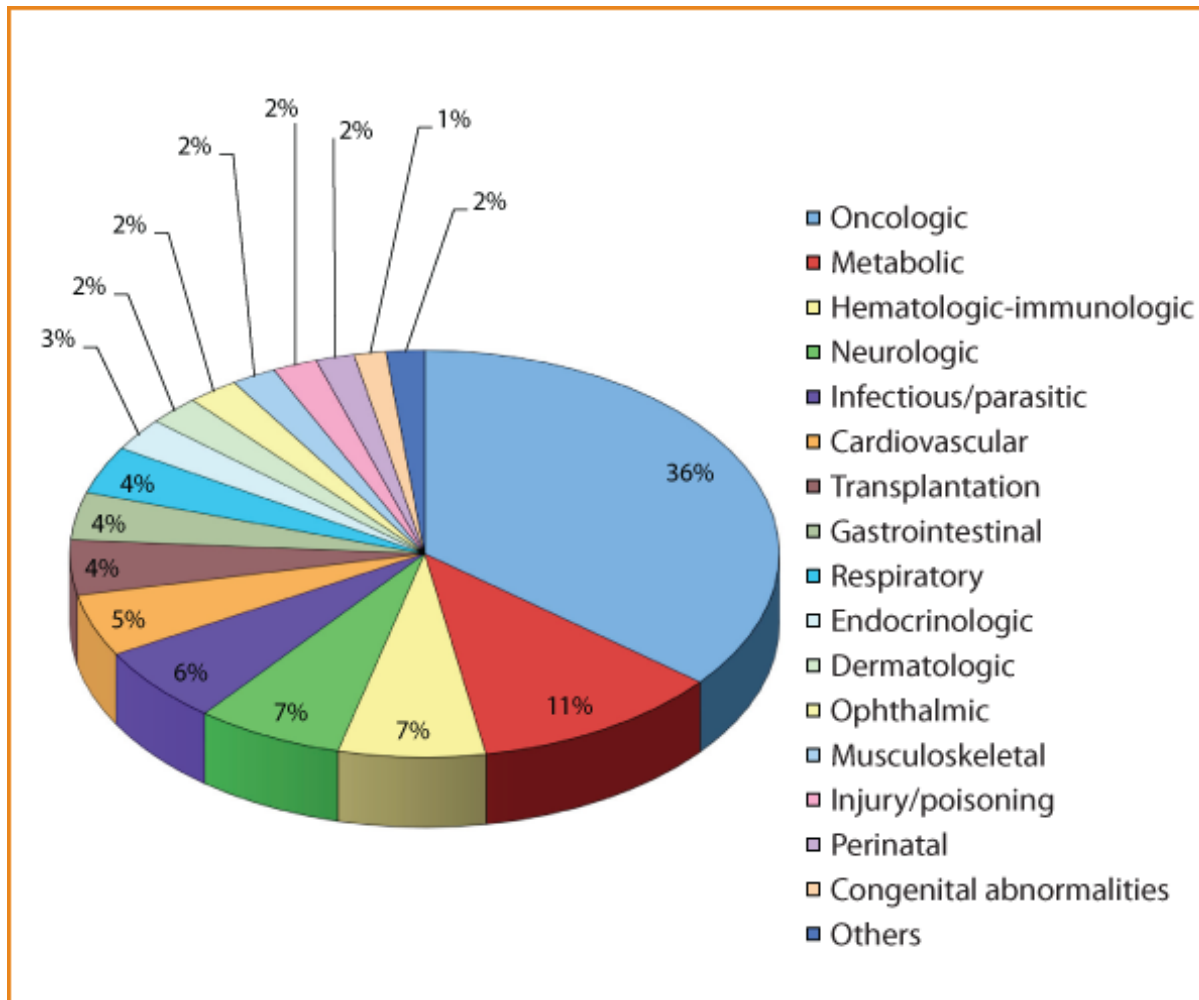
Source: EvaluatePharma[®] 30 September 2015



*excluding generics



Overview of US Orphan and Rare Disease Market









- ~7000 rare diseases
- ~25 million people
- 25% of a product's sales have to be orphan indications to maintain designation
- As of January 2017...
 - 458 ORD products
 - 302 single indication ORD
 - 72 several mass market Indications
 - 84 multiple disease states
- Average therapy cost \$111,820/yr.



(Emotional) Authorization and Step Process – Patient Journey



Products – Understanding Differences in Commercial Models

	Brand	Generic	Specialty	Biosimilar	Orphan	Precision
						
Cost	Low	Very Low	High	High	Very High	Very High
Patient Base	Very large	Very large	Small to Medium	Small to Medium	Very Small	Very Small
Payer Barriers	Low	Very Low	High	High	Very High	Uber High
Complexities	Rare	Very rare	PA, Step Edit, Benefit Design, Reimbursement, Storage, Admin	PA, Step Edit, Benefit Design, Reimbursement, Storage, Admin	Find patient, PA, Step Edit, Benefit Design, Reimbursement, Storage, Admin	Qualify patient, PA, Step Edit, Benefit Design, Reimbursement, Storage, Admin



Why Use Exclusive Networks?

Answer: High Control/High Touch

Channel Access To Drug			Level of Control
Open	Preferred	Limited	Exclusive
<ul style="list-style-type: none"> > 100k patients Low control Low touch service No data visibility No limit on access 	<ul style="list-style-type: none"> 100k – 20k patients ↑ control Low touch service ↑ data visibility No limit on access; list of preferred partners 	<ul style="list-style-type: none"> 20k – 5k patients ↑ control Medium touch service ↑ data visibility 1 SPP/1,000 patients/year 1 SPP/100 new patients/month 	<ul style="list-style-type: none"> < 5k patients High control High touch service High data visibility 1 SPP

These networks can vary by drug AND by type of site of care



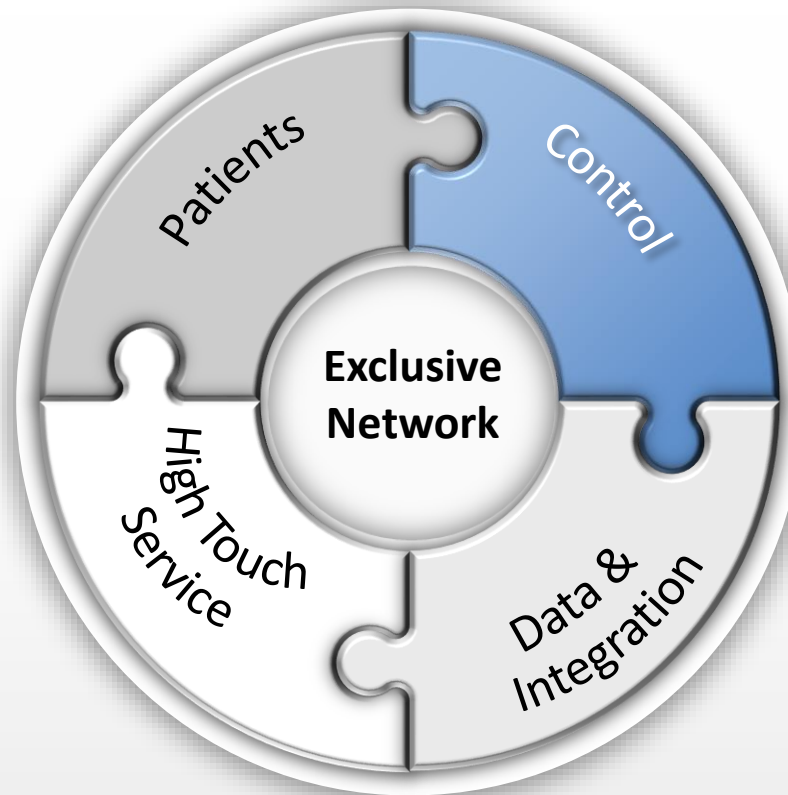
Rationale for Exclusive Network for Orphan / Rare Drugs

Patients

- Small patient population
- With multiple SPPs each could get very few

High Touch Service

- “White glove” – provide holistic patient medication management and assist with payer hurdles
- How to ensure same high touch across multiple SPPs



Control

- Every patient / caregiver counts – can't afford to lose even one
- Every therapeutic intervention matters

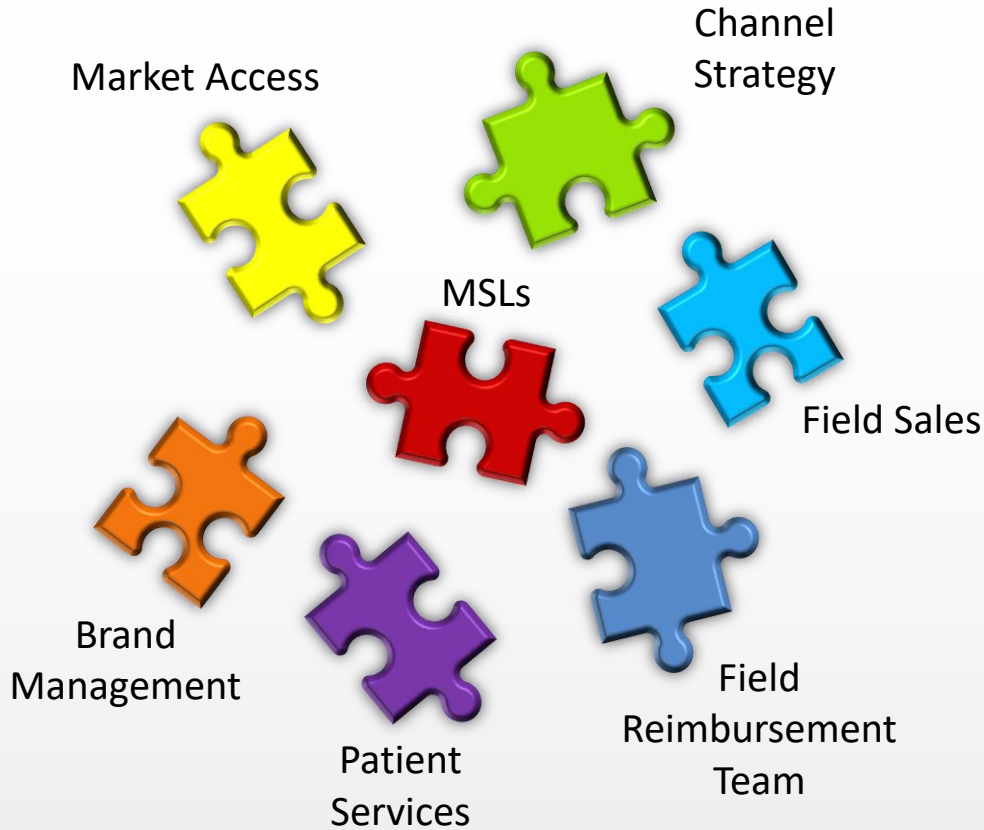
Data & Integration

- Accurate and timely data critical, especially for a 1st launch
- Data integration easier with fewer partners
- Integration with patient services



What do Manufacturers Need to Consider to Put the Puzzle Together?

Bridging Silos



Patient Journey



You Are Going to Have Frustrated Constituents

Payers	Providers	Health Systems	Out of Network Pharmacies
Owned SPP not in manufacturer network	May not be able to use SPP of choice	Usually excluded from exclusive networks due to limited reach	Want in!
May have to contract with SPP outside of their preferred network at higher rates	Perception this is a more complicated process	Have responsibility for patient – how do they have visibility to the entire journey	May have the other medications for these patients and can't fill this one



Winning an Exclusive Network Contract

What **NOT** To Do

- Primarily focus on total revenues and growth
- Accreditations

What To Do

- Deep disease state knowledge
- Empathy with patient
- Empathy with providers
- Payer relations and nuances
- Care coordination
- Willingness to share data
- Willingness to integrate with patient services



Expanding Indications and Network Implications

Networks are morphing to accommodate multiple indications with tailored care

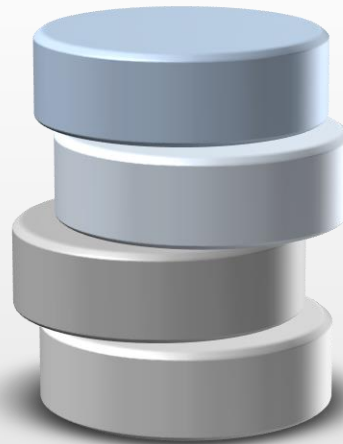
Single Indication

Laser focused



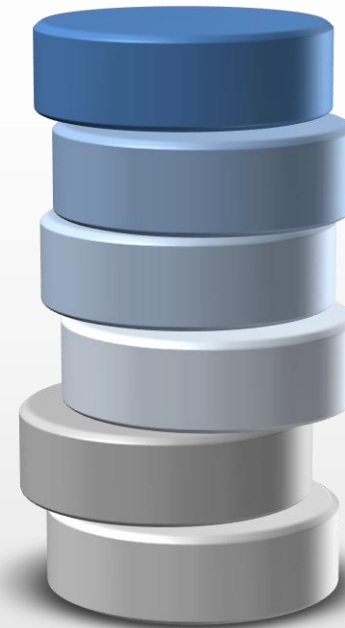
Follow-on Indications

Manage complexity of disease state specialty



Expanded Indications

Networks tailored by disease state, patient, caregiver and provider types



Key Takeaways

Specialty Pharmacy

- Substantial market opportunity
- Winning an exclusive network contract is difficult
- Solid business model
- Key is
 - Empathy
 - Willingness to integrate with manufacturer for care coordination

Manufacturer

- Can't miss a single patient or therapeutic intervention
- Imagine every patient (and their caregiver) is like an egg
- Many channels will want access but they have to prove they are the best at it
- Be VERY careful not to be laissez faire or cavalier with your approach to channel strategy or configuration

You Never Get A 2nd chance to make a 1st impression





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