Amicus Therapeutics

Rare Disease Supply Chains

Patient centric philosophy

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What am I going to talk about?

- Rare disease supply chains, a patient centric philosophy
  - Starting with the patient (understanding the patient needs, size and market dynamics)
  - Decoupling the supply chain, building efficiency and agility
  - Challenges / opportunities of small volumes (Supply Chain)
  - Ending with the patients

Who's up here?

- Father of four and a very understanding wife!
- I don't have hobbies as such, but my passion is my family and work, and by work I mean working in the Rare Disease arena

- 20+ years experience (in Rare Disease) at Genzyme, Genzyme / Sanofi, Synageva and now Amicus
- Not going to talk about Amicus…. Just why I joined them

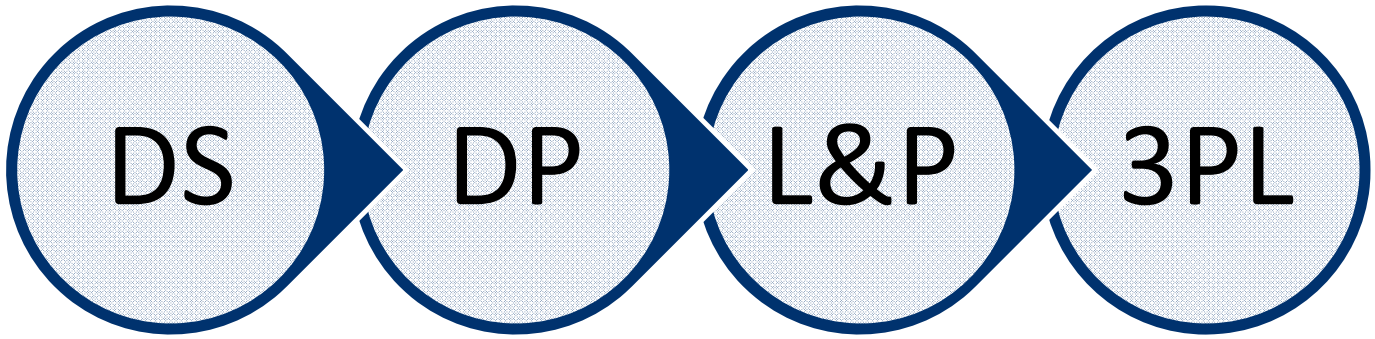
- Launched 9 Rare disease products
- Lived through supply crisis… and the impact on patients

Starting with the patient (understanding the patient needs, size and market dynamics)

- Patient populations (100's / 1000's)
- Small markets (10's of patients)
- Patient population equal small supply requirements equals higher cost (most times)
- This requires a different approach to the designing supply chain around manufacturing capacities and matching the supply v demand
- You truly need to understand the disease and treatable population size as finding an additional 1000 patient (globally) could mean doubling your capacity, which may not be a quick turnaround
- The last mile (getting the drug to the patients) maybe very different for the traditional models

Decoupling the supply chain, building efficiency and agility

- When dealing with rare disease products you approach the supply chain design from scratch
- Traditional supply chain models support multiple hand-off's and touch points and focus on volumes / efficiencies / service
- Rare disease supply chain start with the patient and work upstream through the supply chain focusing on service and agility, removing the touch points
- Patient numbers are small any deviation in demand normally will have an impact on the supply chain so building in late stage customization and flexibility is a key element, the challenge is doing this is a cost effective manner.



Challenges / opportunities of small volumes

Data Management

- The supply chain is complex enough to need good S&OP tools
  - However unless you are division of a large company the does not doesn't justify significant investment in ERP
- The supply base is diverse
  - Often more than a larger pharma company with an internal manufacturing footprint
  - Collaboration tools need to be flexible and low cost
    - Hard to get CMOs to agree given limited spend
- Internal forecasting is sometimes limited or nonexistent
  - This often drives supply chain team to manage in the dark
  - Supply is pushed through the chain
    - Postponement inventory strategy is often single lever available

Challenges / opportunities of small volumes

Supply Chain Redundancy

- Volume aligns to single global sourcing for many items
  - Supply risk is thus managed by large amounts of inventory at many nodes
    - This often leads to expiry management issues
- Secondary sourcing strategy often prohibitive
  - Cost of implementation is often measured in years to pay back
  - Lack of internal focus/desire
  - Can cause loss of supplier interest
    - Volume depletion leads to second class status
- Effective disaster recovery management is complex
  - Reliance is a function of supply base capabilities
- Management of multiple geographies often happens from multiple locations not all in any single geographic region

Challenges / opportunities of small volumes

Staffing

- Internal FTEs are often seasoned generalists
  - Hard to justify subject matter experts
- High reliance on consultant and/or contract staff
  - Often difficult to drive ownership
- Small staff over more than one geography
  - Lack of critical mass
  - Can become siloed
- Cross training difficult
  - Generally limited mobility
- Little if any chance for succession planning

Challenges / **opportunities** of small volumes

The future if bright

- Technology costs continue to drop
  - Cloud based systems allow for a pay as you grow model
  - Software tools are now being “right” sized
- CDMOs are changing
  - Biotech has changed the paradigm
  - Small is the new big
- Inventory is not always the enemy
  - Proper management of investment can allow postpone to be a competitive advantage
- Rare disease supply chains are forced to think about the patients and how best to support them
  - Striving for deliveries to the patient’s home (where appropriate) and providing the support to the patient is where we want to go and the industry should be looking to the retail industry for example of where we can get to in the future…..
- Supply Chain professional training is being viewed as important by C-level leaders
  - Again Biotech has changed the paradigm
    - The change in COGs moving from small to large molecule has opened up a change in view point

- **Most Importantly we get to truly put the patient at the center of what we do**

Conclusion

- Movement away from a one size fits all disease treatment paradigms will lead to a need to be able to manage smaller more customized supply chains

- Rare and orphan disease treatments as well as personalized medicine are focused on customized supply chains and well suited for the future

- More than processes it is the holistic view of the supply chain and the integration into the business with active input and engagement from stakeholder (HCP's and Patient groups) to continuously develop the supply chain to meet the patient needs.

- When thinking about direction / strategy / decision just ask a very simply question;

"Does this benefit the patient?"

Q&A