Market Access & Pricing Challenges & Considerations in Rare Diseases

By Ravi Sodha, PhD, MBA

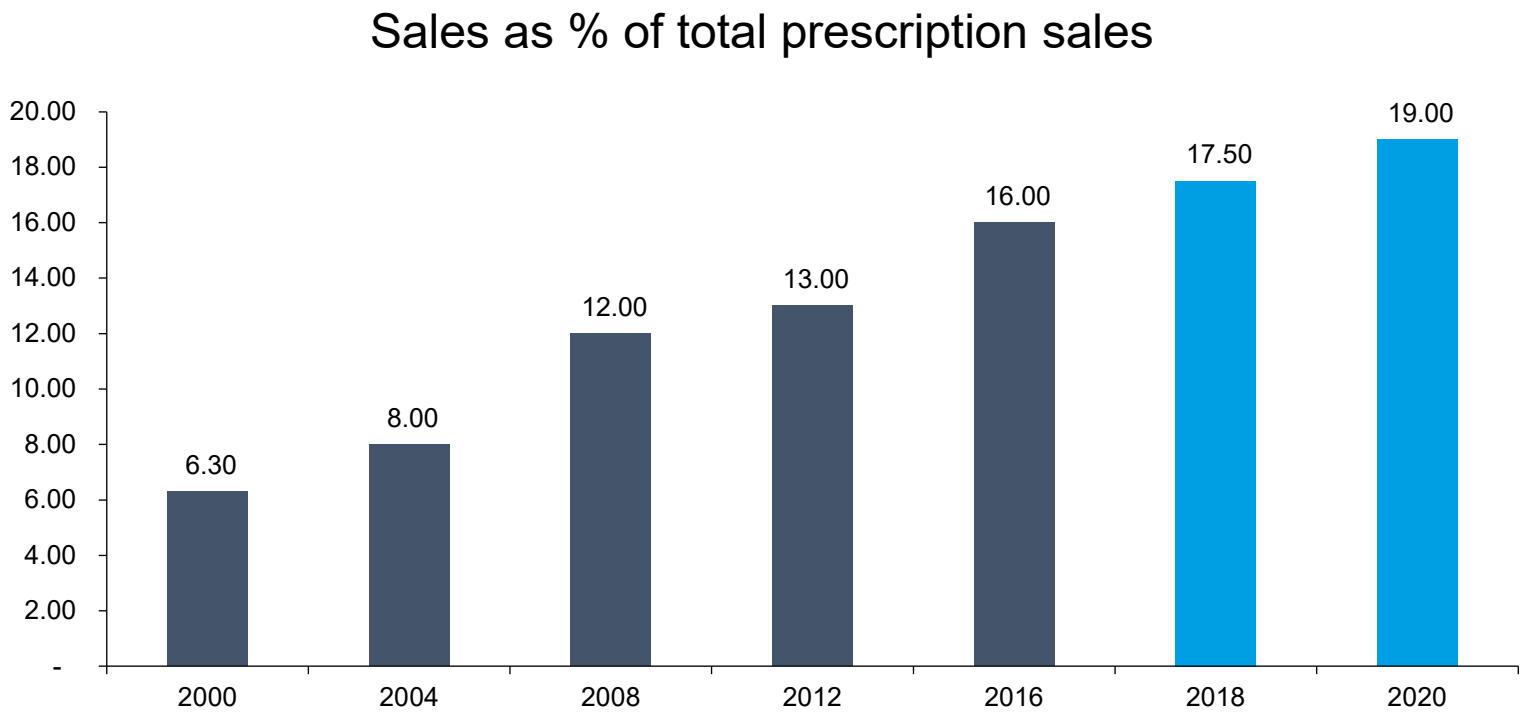
Sodha Pharma Consulting GmbH
ravi@sodha-consulting.com
+41 79 834 61 28

RARE DISEASES – THE FACTS

7’500	Number of rare diseases
1 in 10	people are affected by a rare disease
1 out of 2	patients diagnosed with a rare disease is a child
3 out of 10	children with a rare disease will not see their 5th birthday
8 in 10	rare diseases are caused by a faulty gene
6 – 8 years	is the average time to get a confirmed diagnosis
95%	of rare diseases lack an FDA approved treatment
FDA	approvals for rare diseases lag considerably behind rare disease designation

Source: http://www.globalgenes.org

THE ORPHAN DRUG MARKET IS GROWING



Source: Evaluate Pharma, Orphan Drug Report, 2017

MARKET ACCESS - ISSUES

- Mostly, only sparse clinical data available.
  - Regulatory agencies and payers require more data, larger studies, “value for money”
- Patients are now more “therapy savvy” and global.
- Usually, companies enter relevant therapeutic area for the first time, so no previous experience or knowledge of disease.
- Competition - more Pharma companies are interested in developing rare disease drugs.
- Careful that the drug prices are not disproportionate to the clinical efficacy.

CHALLENGES TO PRICING – MORE PAYER SCRUTINY

- Patient advocacy groups and patient support groups typically support R&D, legislations, bring patients together, etc. They do not negotiate price.
- Payers have up to now paid up, but there may be a breaking point as prices go up.
- Patient co-pay has gone up
- Use of prior authorization has increased
- Payers may determine value based on cost-effectiveness and unmet need

MARKET ACCESS AND PRICING FORMULA FOR SUCCESS

- Companies often focus on just one orphan indication and one market. Should seek other indications and other territories
- Companies must provide drug's costs and benefits for the health system, caregiver and support systems, and providers and patients
- Identify patients quickly
- Provide strong patient advocacy and patient education
- Equally important is manufacturing and supply chain

STRATEGIES FOR IMPROVING REVENUE POTENTIAL

Pathways	Primary Indication	Subsequent indication	Examples
Early Access	Orphan disease A	Non-orphan disease B	Avastin Bevacizumab
Multiple OD	Orphan disease A1	Orphan disease A2	Glivec Imatinib
Sub indications	Non-orphan disease A	Orphan disease B	Erbitux Cetuximab

SUMMARY

- In spite of new drugs to treat rare diseases, there are still many rare diseases for which there is no treatment
- Most countries provide incentives to develop drugs for rare diseases.
- The ROI in orphan drugs typically exceed that in non-orphan drugs
- Patient support and advocacy groups play an important role in development of drugs for orphan indications
- There are challenges to patient access as these drugs are very expensive
- In spite of more payer scrutiny these drugs are still made available to patients

Thank you

Please reach out for any questions

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