Strategies to Overcome Development Challenges for Small and Medium Sized Companies

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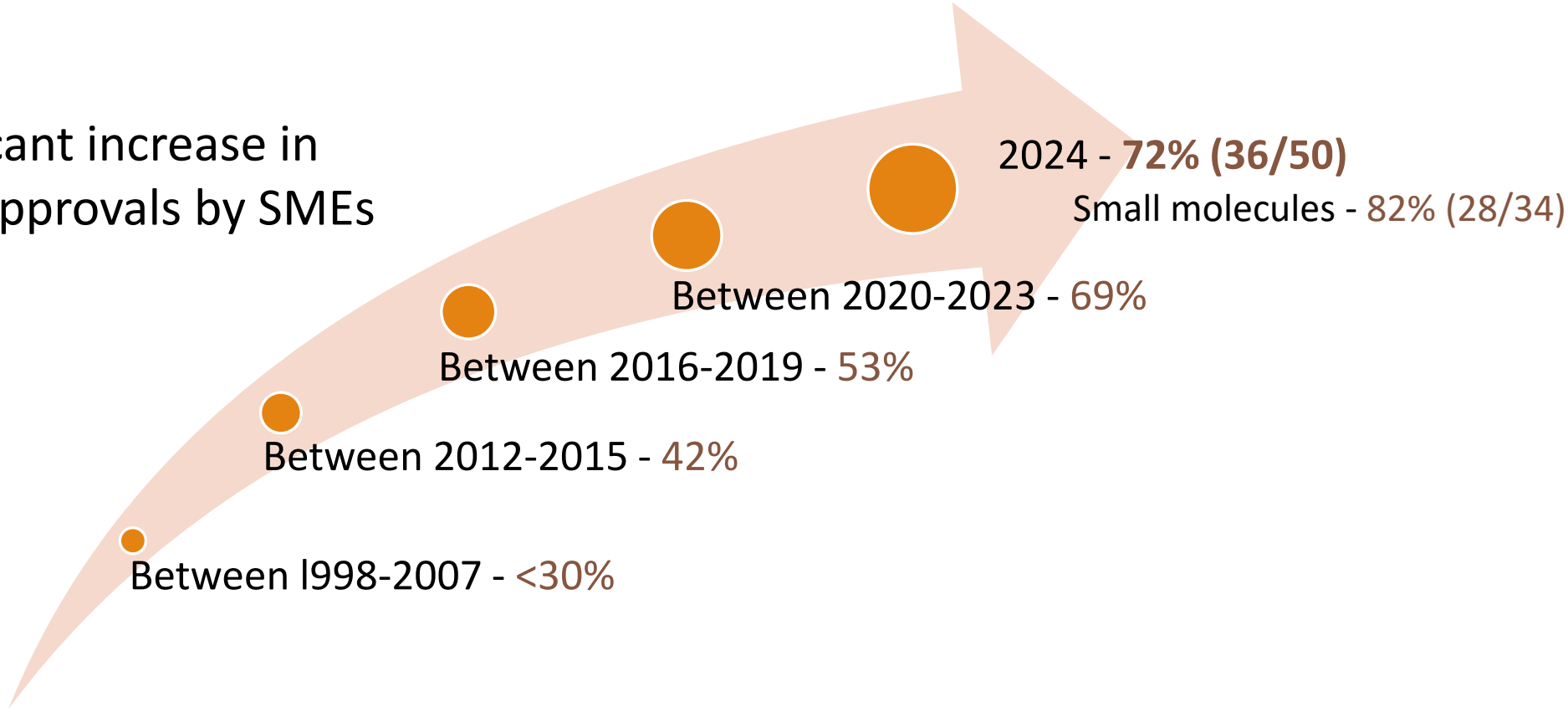
Agenda

- Importance of small-and medium-sized enterprises (SMEs) in Innovation
  - Current trends
  - Contributing factors for growth
- Dynamics of Drug Development for SMEs
  - Challenges and Common Pitfalls
- Strategies to navigate challenges for long-term success
  - Importance of partnership
  - Effective management of third-party vendors

Evolving Landscape of Drug Development

Significant increase in
Drug Approvals by SMEs



Source: US Food and Drug Administration's center for Drug Evaluation and Research; Register of Innovative Drugs – Health Canada
https://www.sciencedirect.com/science/article/abs/pii/S1359644623003823

Key Factors Driving Innovation by SMEs

Agility & Focus

- Nimble
- Focus on niche area / rare disease
- Lack of bureaucratic hurdles

Technological Advancements

- Adoption of AI/ML
- High throughput experimentation

Collaboration with Big Pharma

- SME -> innovation
- Large Pharma -> funding and commercialization resources

Shift in R&D Models

- Focus on targeted therapies
- Align R&D with capabilities

Regulatory and Market Dynamics

- Change in regulatory framework
- Defined/ changing market demand

Challenges in Drug Development for SME

Financial Constraints

High Costs of R&D

Funding Challenges

Cost of Clinical Trials

Regulatory Hurdles

Complex Regulatory Environment

Approval Timelines

Compliance Costs

Limited Resources

Human Resources

Infrastructure

Partnership and collaborations

Market Competition

Competition with Big Pharma

Intellectual Property issues

Market Access and Distribution

Technological Challenges

Access to cutting edge technology

Data Management

Innovation vs. Feasibility

Strategic collaboration with Third-Party Vendors for long-term success of SMEs

Access to Specialized Expertise

Clinical Trials (CROs)

Advanced Manufacturing (CDMOs)

Regulatory Operations and Compliance (COs)

Cost Efficiency

Established Infrastructure

Defined processes and practices

Flexibility and Scalability

Dynamic Operations

Scale up or down aligned with project needs

Accelerated timelines

Streamline various stages of development

Faster progress towards approval and market entry

Aligning CMC planning with Regulatory Requirements for Commercialization

- Implement a risk-based CMC strategy
  - Perform quality risk assessment
  - Develop a plan & share with regulators ahead of time and throughout the development process
  - Track quality by design (QBD) elements to align risk expectation, mitigation strategies and contingency planning
- Explore and employ ways to shorten the traditional approach of regimented sequence of events in a chronological order
  - Perform Formulation Optimization in parallel to registration batches instead of before (during stability monitoring of registration batches)
  - simplify wherever possible
  - Employ predictive modeling to reduce API consumption
  - Make use of AI: target identification, candidate selection, formulation development, navigate regulatory landscape
- Form good collaboration with CDMOs

Addressing Scale, Process and API challenges

Scale Challenges

- Plan scale-up activities at least 2 yrs ahead
- Use already approved manufacturing lines and sites
- Utilize materials from existing approved products
- If using small batch line, choose one from previously approved clinical products

Process Challenges

- Develop Risk based Plan and share with Regulators
- Minimize tech transfer by having consistency in team/ people and goals
- Lock-in early to minimize risk
- Use predictive tools where possible to minimize API usage

API Challanges

- Use API for formulation/ process understanding and clinical supply support
- Aim for multi-batch API production to reduce risk rather than one batch per year
- Finalize the solvent for API manufacture early and ensure it meets global acceptability for residual levels
- Coordinate API and drug product supply chains to mitigate risks in new supply manufacturing

Case Example: Collaboration with third parties for CMC - Path → Commercialization

- Collaboration with a peptide manufacturer with global presence and expertise for:
  - Streamlining the downstream manufacturing process and purification steps
  - Tech transfer and scalability to ensure readiness for commercialization in multiple jurisdictions
  - Assay development to identify and measure unknown impurities
  - Comprehensive risk assessment to avoid time consuming and costly cold chain transport validation studies for drug substance
  - Ensure materials are globally acceptable (US/EU/JP) and meet all country standards
- Collaboration with an established third-party entity in Canada for:
  - Making use of existing licenses/approvals, i.e., Drug Establishment License (DEL) and corresponding Quality Systems for importing drug product into Canada

Enhancing Success in Clinical Development

- Tailored selection and partnership with the Contract Research Organization (CRO)
- Embracing cutting-edge technologies and data analytics to optimize trial execution and outcomes
- Flexibility and adaptability to be prepared for unexpected challenges and having contingency plans in place
- Efficient and effective patient recruitment and retention strategies
- Ensuring that data is collected accurately, stored securely, and analyzed effectively
- Implementing a robust Quality Management System (QMS) will ensure adherence to industry standards, compliance with regulatory requirements, and consistently high-quality deliverables.
- Ensuring that all aspects of the trial comply with regulatory requirements

Case Example: Collaboration with third parties for Clinical development

- Collaboration with key academic leaders as collaborating investigators for:
  - Identification of comprehensive stroke hospitals/units globally
  - Quality conduct of global clinical trials while maintaining the local/regional standards of stroke care
- Collaboration with an experienced clinical supply chain vendor for:
  - Clinical labelling, packaging, logistics and global distribution
  - Management of randomization and blinding
  - Supply chain optimization for inventory management and forecasting to reduce waste
  - Leveraging experience managing cold-chain requirements

Managing Regulatory Expectations and Ensuring Compliance

Meet with Regulators Frequently AND Consult with Regulatory Experts

Understanding Regulatory Landscape Guidance on Regulatory Requirements

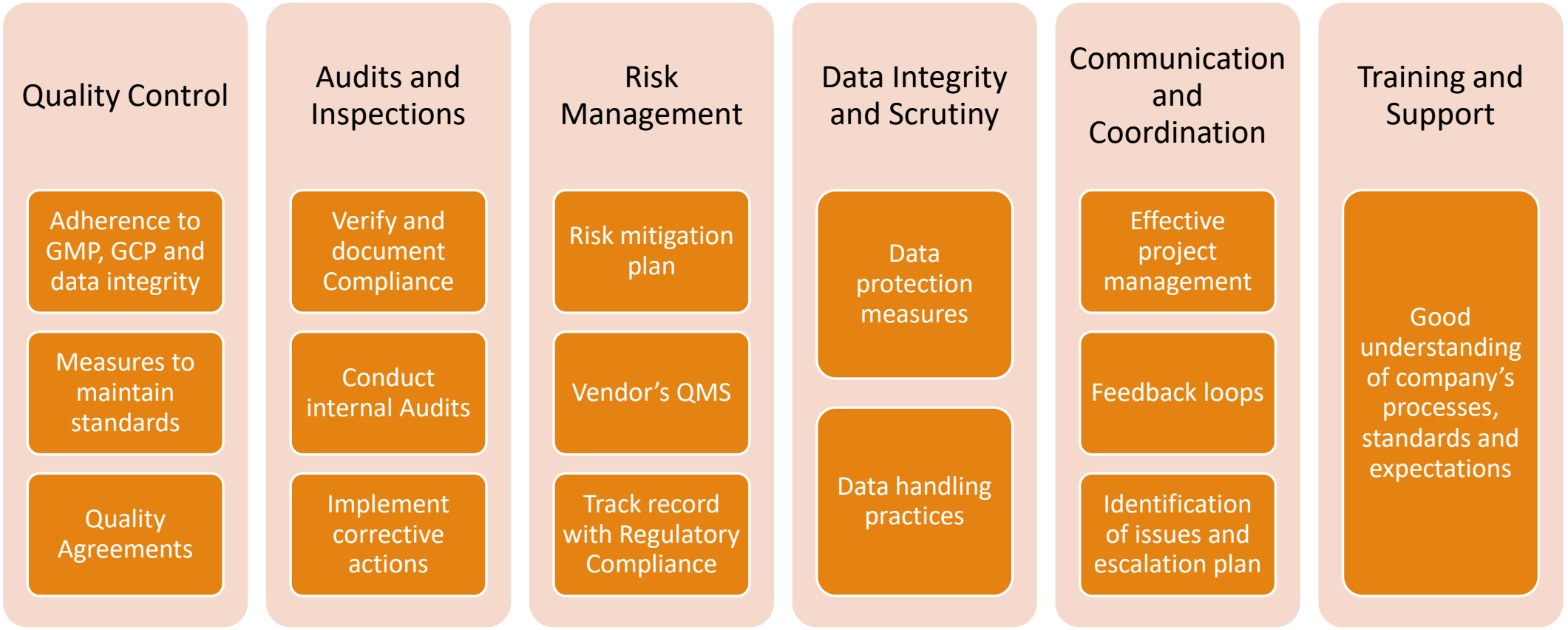
Risk Mitigation

Explore Expedited Pathways

Building Trust and Transparency

Efficient Clinical Trial design

Oversight and Management of Third-Party Vendors



Successful Drug Development requires Multifaceted Approach

Leveraging regulatory incentives

Building strong value propositions around the critical/unmet medical need

Engaging with patient advocacy groups regarding drug's safety and efficacy

Developing a comprehensive CMC strategy that enables a smooth transition from the development phase to the commercialization

Addressing the complexities of regulatory filings and ensuring compliance with regulatory guidelines

Targeted and Effective collaborations for development, funding & relationships (investors, partnership with big pharma, CRO/CDMOs, patient advocacy etc.)

Thank You !