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Lecture 6 The Regulatory Landscape

Who I am…

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Regulation of Healthcare Markets

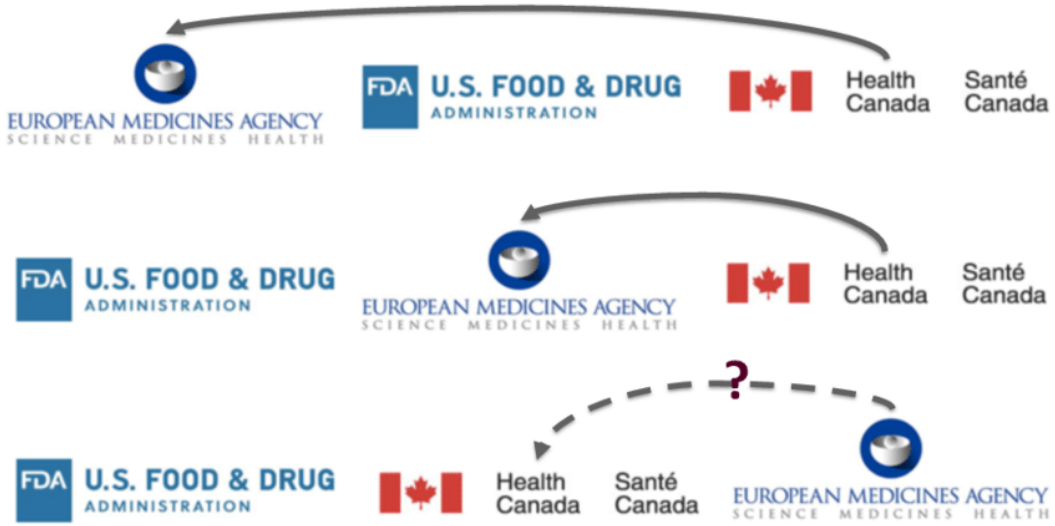
Global Regulatory Bodies

Mandating the regulation of pharmaceuticals, medical devices, and vaccines

FDA – Food and Drug Administration – USA

EMA – European Medicines Agency – EU

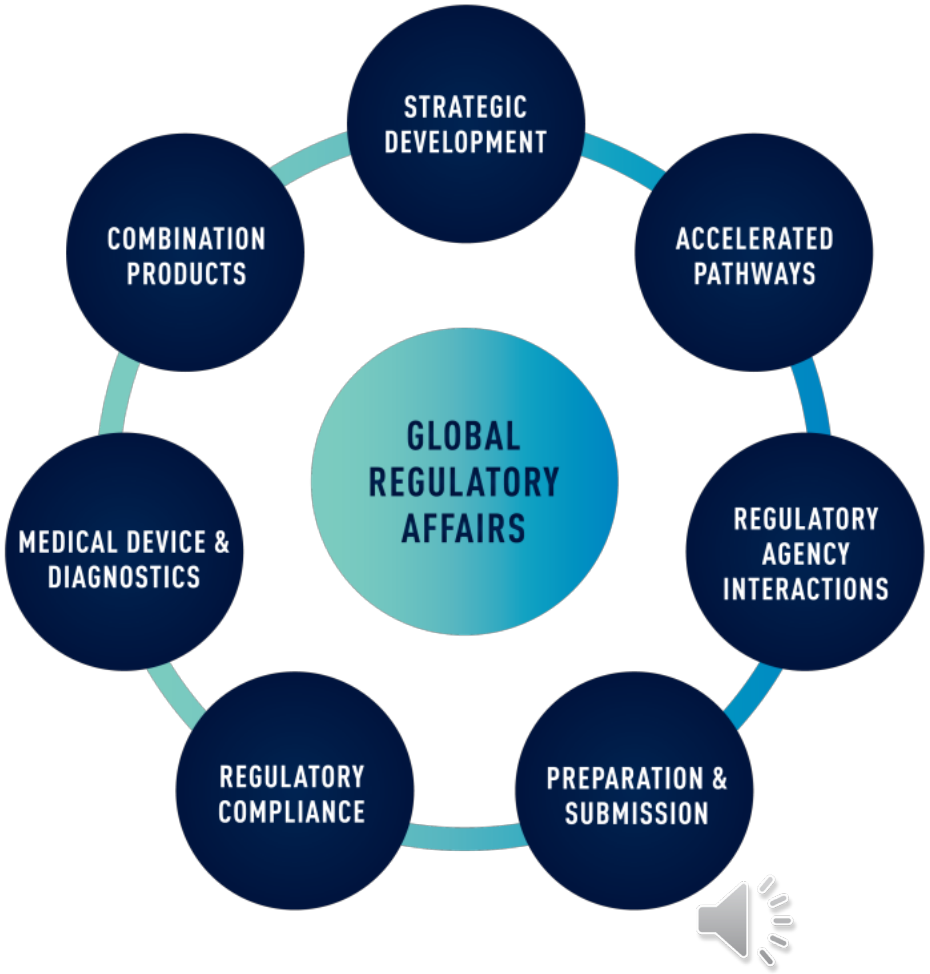
Health Canada - Canada



Regulatory Affairs

Profession dealing with organizations adherence to regulatory compliance

- Necessary for almost all biotechnology companies despite their maturity state



Drug Regulation

The Regulatory Pathway

Preclinical Testing – Laboratory/Animal studies for safety

Clinical Trials – Human testing (Phase I, II, III)

Market Authorization – Approval by regulatory body (FDA, EMA, Health Canada)

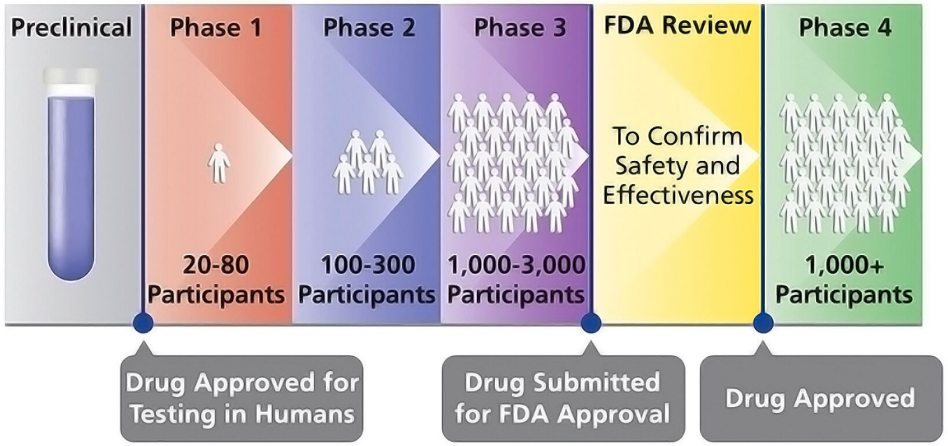
- Stage IV clinical trial can occur post-approval, testing long term effects

Preclinical Testing

Evaluating the biological activity, efficacy and safety prior to human trials

- 2 main measures: **Pharmacokinetics** and **Pharmacodynamics**
- Adherence to **Good Laboratory Practice** (GLP) standards
- Relatively cheap compared to clinical trials
- Require solid study design that can be carried forward

Clinical Trials



Phase I Trials:
Objective: Assess safety and dosage.
Participants: Small group of healthy volunteers.
Focus: How the drug is metabolized and its side effects.

Phase II Trials:
Objective: Test efficacy and further evaluate safety.
Participants: Larger group with the disease/condition.
Focus: Short-term side effects and how well the drug works.

Phase III Trials:
Objective: Confirm effectiveness, monitor side effects, compare to commonly used treatments.
Participants: Large group of patients.
Focus: Gathering more information about safety and efficacy, studying different populations and different dosages.

Market Authorization

NDS – New Drug Submission – Required by Health Canada for market approval of all drugs

NDA – New Drug Application – Required by FDA for market approval of small molecule drugs

Keys for Successful Market Authorization

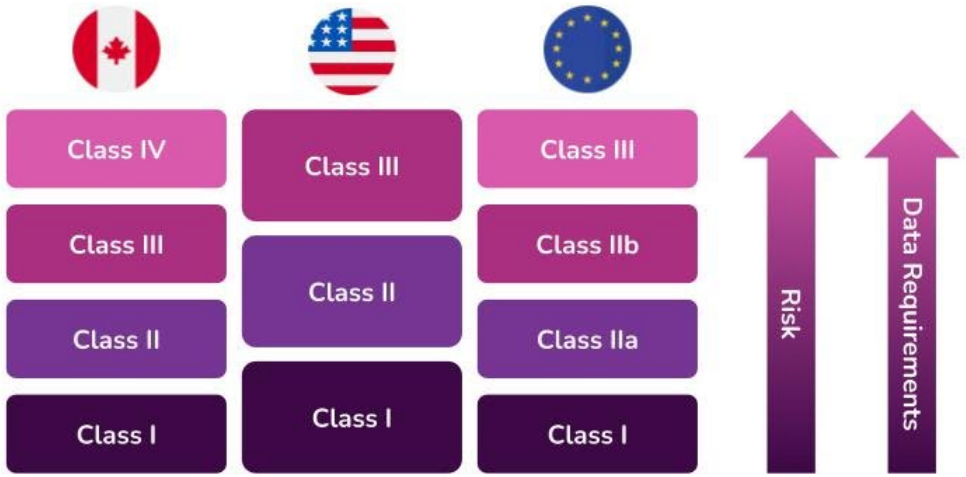
Drugs that are approved emulate the following criteria:

- Comprehensive data (clinical and preclinical trials)
- Clear and accurate labeling (understanding of side effects/risk)
- Manufacturing Consistency
- Regulatory Compliance
  - Your regulatory affairs employee must do a good job!
- Risk-Benefit Analysis
  - HTA, Value proposition, etc.

Medical Devices

Medical Device Classes

Medical devices can be separated into classes, based on their level of risk



Class I – Toothbrush
Class II – Pregnancy test kit
Class III - Contacts
Class IV – Pacemaker

The Regulatory Pathway – Medical Devices

Clinical Testing – Human trials for device efficacy and safety

Premarket Approval (PMA) – Approval for device manufacturing

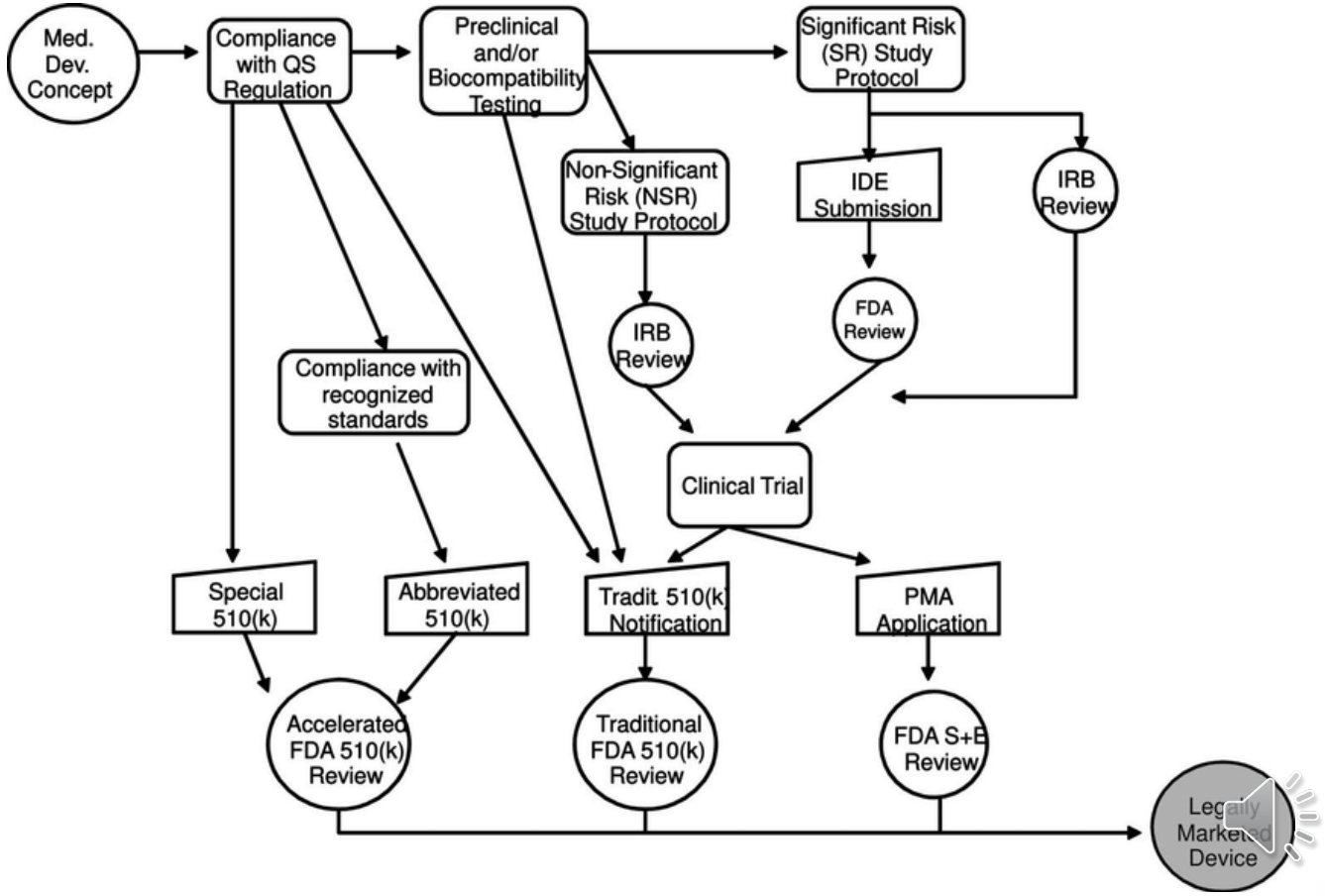
Quality System Regulations (QSR) – Manufacture regulation

Market Authorization – Approval by regulatory body (FDA, EMA, Health Canada)

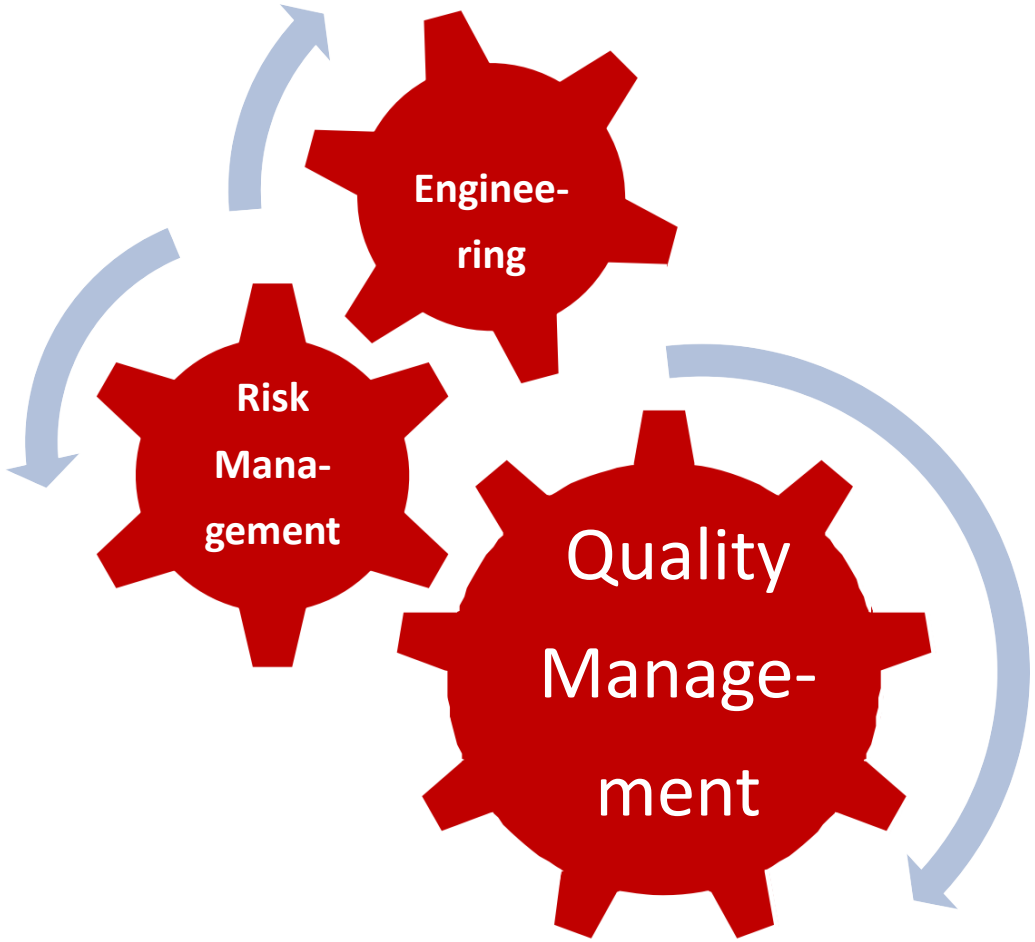
- Stage IV testing can occur post-approval, testing long term effects

Medical Device Regulation is Complicated!

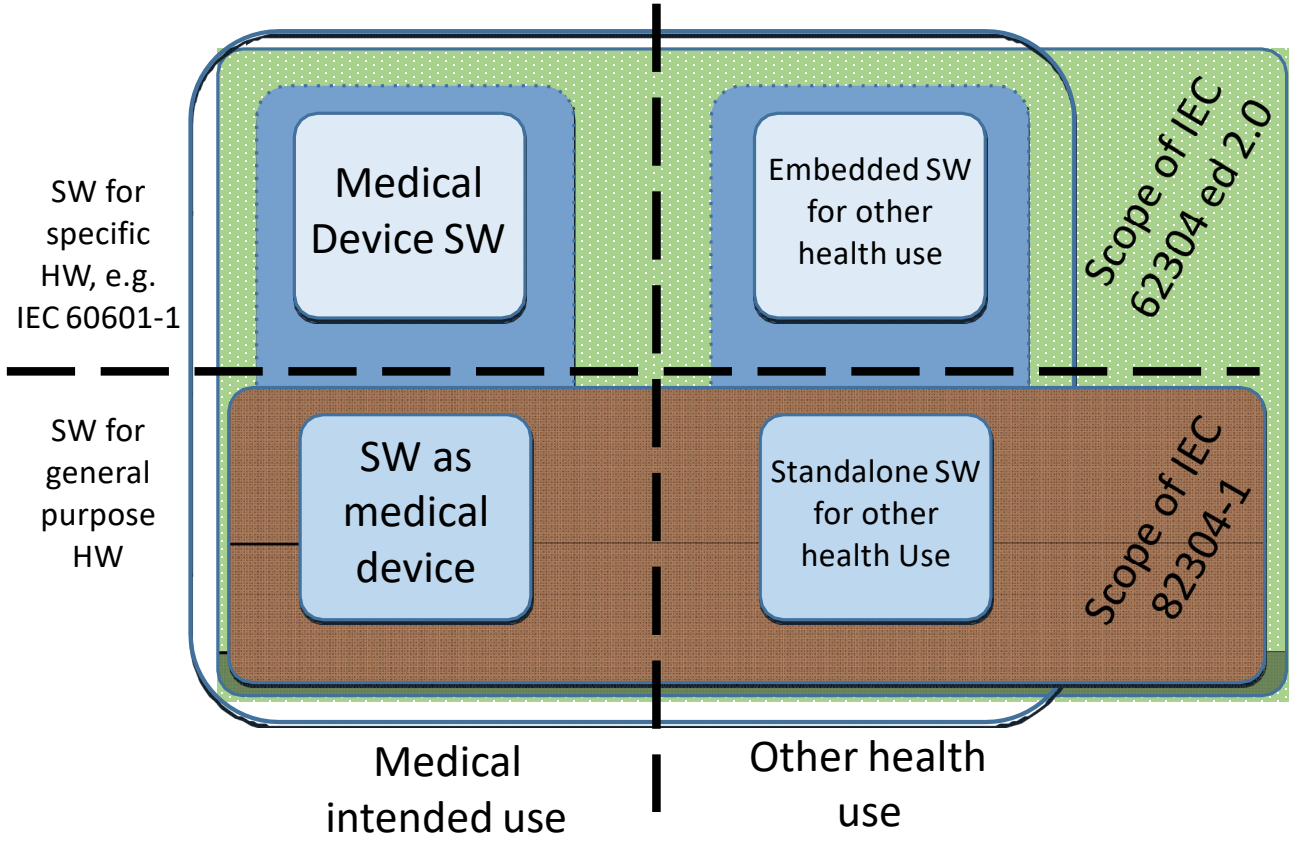
- Medical devices do not follow a linear regulation path that drugs may follow
- Heavily dependent on the device class and country



Three basic principles



Software as part of a MD vs Software as a MD



Company: ISO 13485 (QMS)

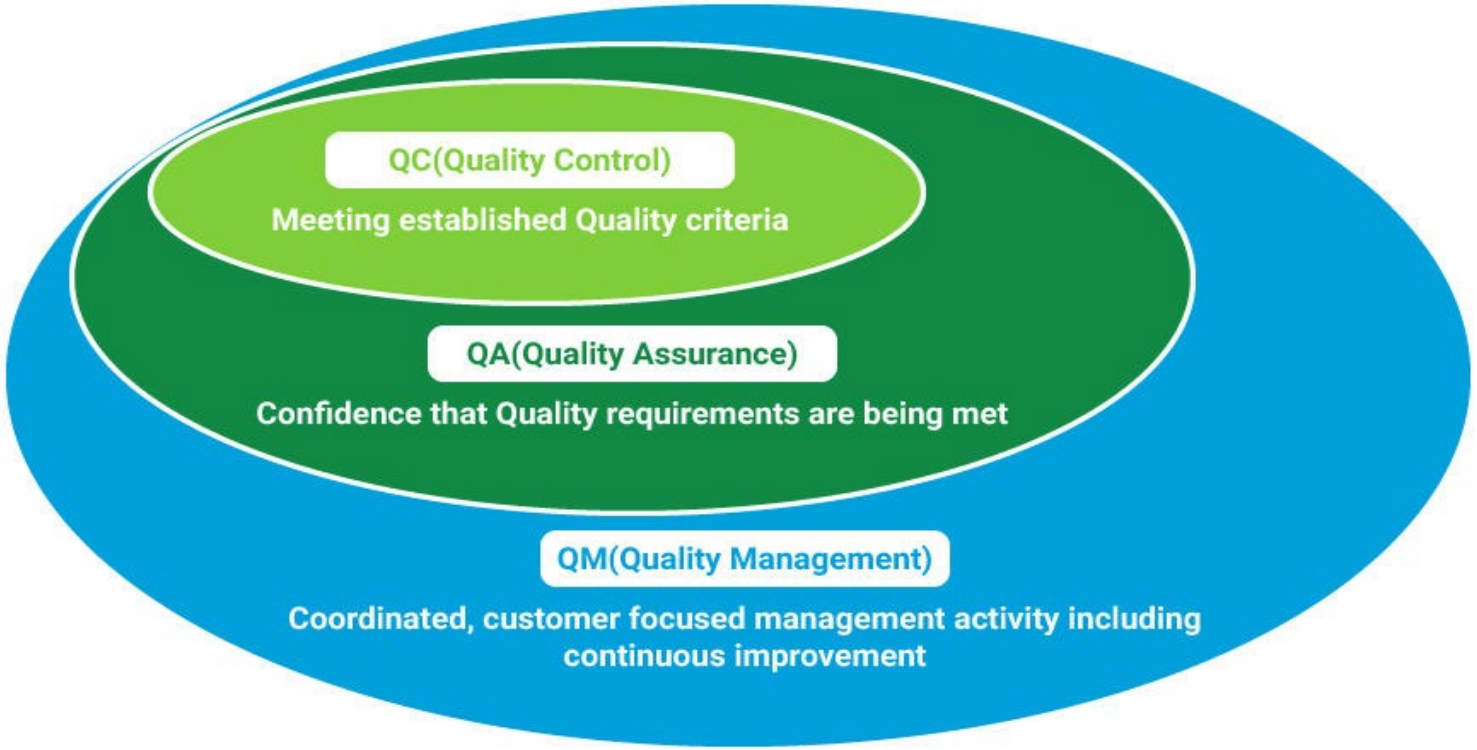
Product:

- IEC 82304-1 (SaMD) or IEC 60601-1 (PESS)
- ISO 14971 (Risk management)
- IEC 62366-1 (Usability)

Software: IEC 62304 (SW process)

HEALTH SOFTWARE - software intended to be used specifically for managing, maintaining or improving HEALTH of individual persons, or the delivery of care

A Quality System is defined as the organizational structure, responsibilities, processes, procedures and resources for implementing quality management.



Stage IV Market Surveillance

Novel concept, investigating the performance of a drug or medical device while in market

- Real World Evidence (RWE)
- Observe long term adverse effects and unforeseen risks

Drugs – Focus on Pharmacovigilance

Medical Devices – Focus on performance

Stage IV Market Surveillance - Ozempic

Completely hypothetical example of Stage IV

Objective of the Trial:

- To evaluate long-term safety and efficacy of Ozempic (semaglutide) in a real-world setting.
- Monitoring for any delayed adverse reactions or benefits.

Building Relationships with Regulators

Schedule regular meetings or check-ins with regulatory representatives

Engage in workshops or training provided by regulatory bodies

Develop clear and concise communication materials for submissions and queries

Participate in industry forums or regulatory conferences

Establish an internal point of contact

- Regulatory affairs associate

Regulatory Challenges in Medical Sciences

Allocate budget specifically for regulatory compliance and consulting

- On top of R&D, money has to be set aside to help progress the approval pathway

High failure rates, long time constraint with approvals

Use regulatory intelligence tools to stay informed about relevant guidelines.

- Online databases and resources to monitor region-specific regulatory changes

Minimizing your Regulatory Roadblocks

Perform risk assessments to identify potential regulatory issues.

Arrange pre-submission meetings for guidance.

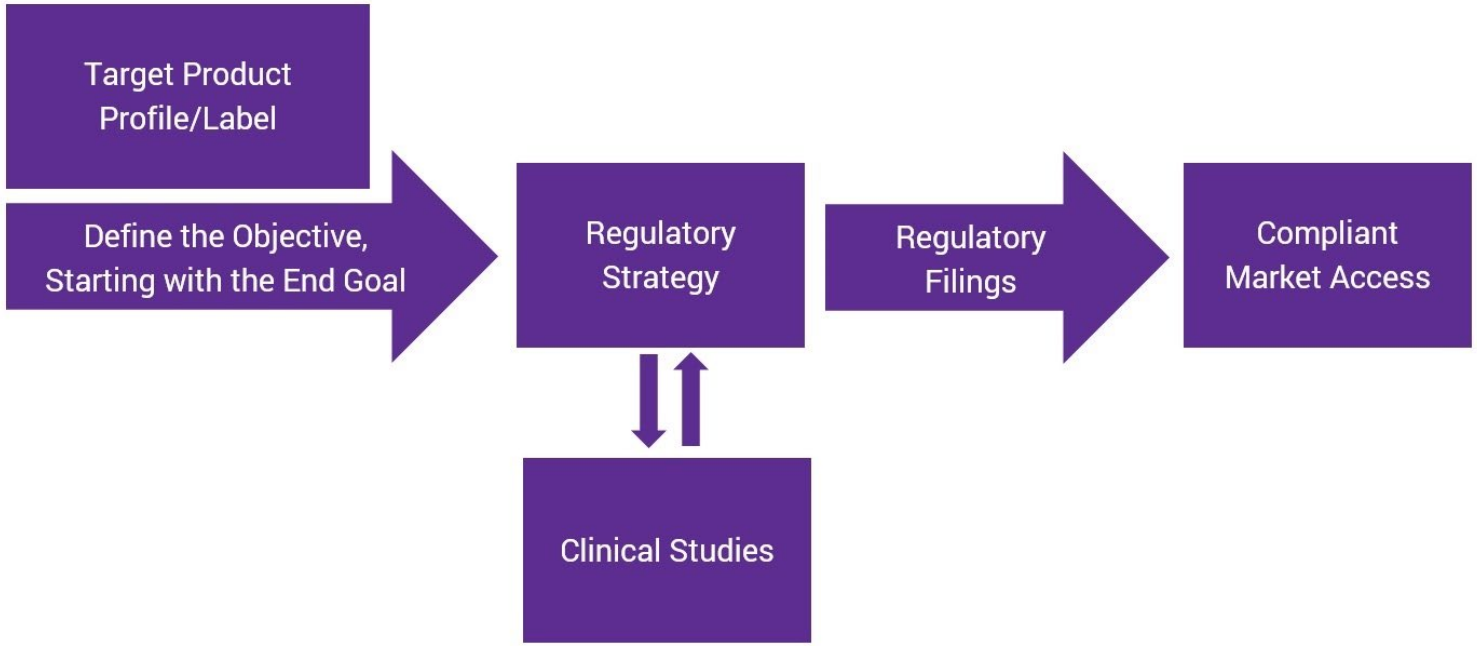
Strengthen quality assurance processes.

Regularly update SOPs based on regulatory changes.

Implement data management tools for efficiency.

Regulatory Strategy Trends

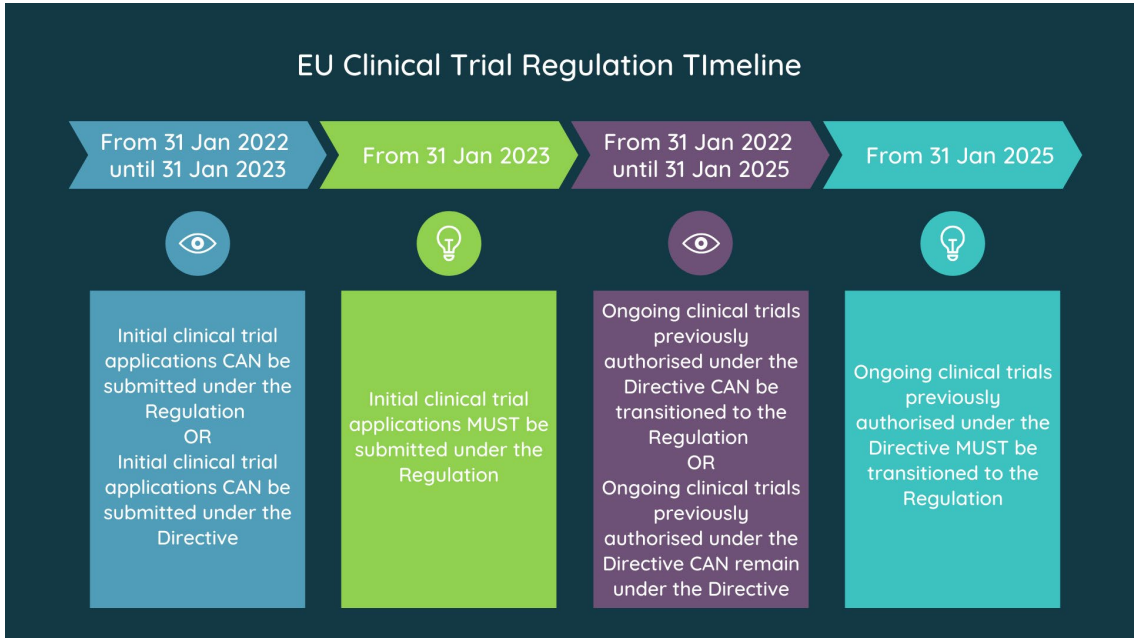
With all aspects of business, regulatory affairs have specific strategy based on changes in regulation per region



EU Clinical Trial Regulation

This new system harmonizes and simplifies the clinical trial process in the EU.

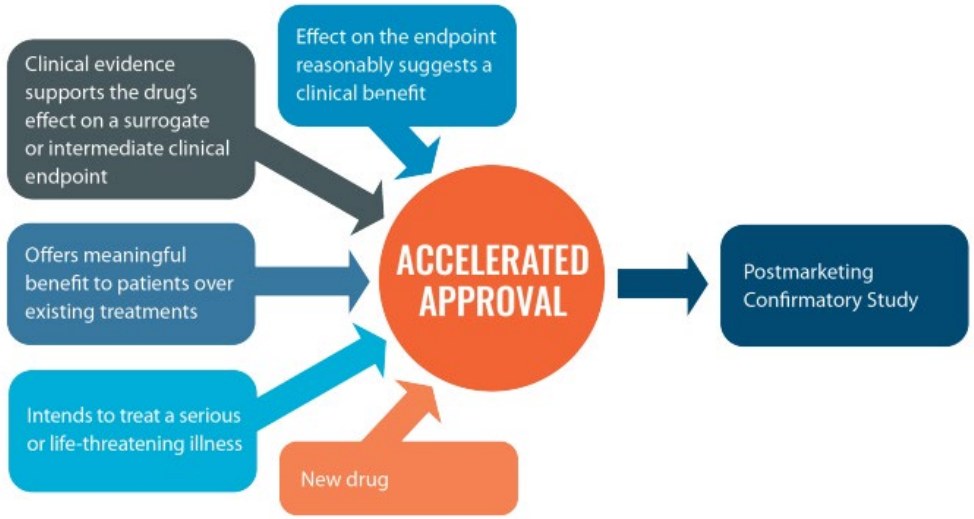
Strategy: Companies should familiarize themselves with the Clinical Trials Information System (CTIS) for streamlined submissions and transparency.



FDA Focus on Accelerated Development

The FDA is emphasizing rapid development of complex therapeutic products.

Strategy: Utilize the FDA's pilot programs and initiatives, like the Chemistry, Manufacturing, and Controls (CMC) review pilot, for expedited product development.



Project FrontRunner Initiative by FDA

Encourages early-stage development of new cancer therapies.

Strategy: Focus on developing therapies for advanced diseases in earlier clinical settings, considering the potential for quicker access to innovative treatments.

End of Lecture 6

That's it!