HealthTech Activator

Regulatory Touchpoint Map Pharmaceutical Drug Product and Biologics – US FDA

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POWERED BY
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New Zealand's Innovation Agency

REGULATORY TOUCHPOINT MAP PHARMACEUTICAL DRUG PRODUCT AND BIOLOGICS – US FDA

PERSPECTIVE OF PERSON/ ROLE	KNOWLEDGE REQUIRED TO OPERATE EFFECTIVELY	RELEVANT REGULATORY FRAMEWORKS	NOTES
SEED			
Scientist/inventor	Is product idea/technology regulated?	• Part of Freedom to Operate analysis	• Specific to each country • Understand trade-off between achieving regulatory compliance and altering product's intended use e.g. often lower value and lower barrier to competitors for non-regulated products • Understand that work may have to be repeated in the future for submission purposes if compliance is not established
	Which regulations/standards potentially apply?	• Relevant guidance documents • Relevant standards • Pre-IND • IND • NDA/BLA • GMP • Good Clinical Practices • Good Laboratory Practices • See FDA definitions of • Drug • Vaccine, blood and biologics • Biosimilars • Combination products • Generic	• Establish regulatory strategy • Demonstrate reproducibility of the technology at a commercial scale • Differentiate clearly between drugs and devices – not always intuitive e.g. Is your technology a combination product? • Early engagement with regulators to establish submission route, data requirements and clinical track (if applicable) with regulatory and technical experts • Establish science, proof of concept • Is the technology reproducible in multiple controlled environments? • Perform product testing to ensure output meets pre-determined specifications
	Timing of regulatory requirements	• Early requirement to: • Establish proof of concept • Establish protocol driven studies (in-vitro and in-vivo testing) • Generate, collect and review data from protocol driven studies • Ensure study design, conduct and record keeping align with regulations	• Aim to address scientific feasibility early, align research with regulatory requirements: • Product description • Target identification • Mode of Action • Characterisation • Formulation • Dose range
	Likely costs and skills needed to pass regulatory science hurdle	• Seek external expert advice for budgeting, process and resources	• Timing of costs important to establish capital runway to support R&D

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SEED			
	Likely costs and skills for regulatory support	• Seek external expert advice for budgeting, process and resources	• Timing of costs important to establish capital runway to support R&D
Medsafe website listed consultants, Tech Transfer Office (e.g. VicLink, LSNZ, CMDT, WREDA)	Where to seek expert (light touch) advice?	• Expertise in drug development e.g. some FDA knowledge may lie within some university departments and research institutes • Medsafe list of professionals	• Form the right team for where the product is in its lifecycle • Limited knowledge of end-to-end regulatory pathways in university, so identify gaps and source expertise, i.e. establish/revise regulatory strategy
	Do multiple regulations apply?	• Do regulations concerning combination products apply?	• Convergent technologies may have primary and secondary regulations, e.g. genetically modified organism expressing new biological therapy (antibodies etc)

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STARTUP, EARLY STAGE			
Founder (technical)	Clear regulatory strategy	• Early engagement with regulators • Relevant guidance documents • Relevant standards • Pre-IND • IND • NDA/BLA • Protection of Human Subjects • Institutional Review Boards • Good Laboratory Practices	• Establish regulatory strategy, revise as needed to meet changes in the business • Regulations apply throughout the lifecycle of the product • Research studies • Safety and toxicology of the substance & product • Product development • TPP • Quality Control testing (substance & product) • Manufacturing and Process Controls • Clinical trial requirements (if applicable) • Proof of safety and efficacy of the final product
	Where to seek expert regulatory advice?	• Medsafe website listed consultants • Local and international experts • Partners • CMOs	• Confirm regulatory strategy prior to research budgeting
	What research studies (pre-clinical) are required?	• Relevant guidance documents • Relevant standards • Apply regulatory compliance or principles from the beginning	• Match reg requirements with potential multinational commercial requirements (e.g. biomarkers and disease models that industry prefer) • Confirm market strategy • In the absence of regulatory requirements, business risk should be mitigated
	What laboratory practices, facilities and standards are required?	• Basic Quality System, Good Laboratory Practice (GLP), Good Documentation Practices (GDP) to maintain traceability and record keeping	

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STARTUP, EARLY STAGE			
Tech Transfer Office (e.g. Callaghan, VicLink, LSNZ, CMDT, WREDA)	Optimal reg pathway for speed of approval and IP portfolio	• Identify options	• Establish/revise regulatory strategy • Be knowledgeable of regulatory pathways, e.g. › fast-track approval, Orphan Drug, priority review › IP protection for rare diseases • Check and balance: commercialisation strategy with IP strategy with regulatory strategy
	Where to seek expert advice?	• Medsafe website listed consultants • Local and international experts • Partners • CMOs	• Confirm regulatory strategy prior to research budgeting
	Is the development team capable of designing and delivering critical regulatory science requirements in a commercial setting?	• Fill gaps in-house or as external consultants	• Do issues of performance, scale and reproducibility exist for the technology?
	How does regulatory strategy and timeline impact commercialisation strategy?	• Align regulatory planning with market launch planning	• Is the company infrastructure experienced and/or adequate to support meeting regulatory and technical objectives?
	How does Regulatory strategy and timeline impact IP strategy?	• Optimise patented time on market and data exclusivity	
Investor (or TTO)	Ensure runway and budget will achieve critical regulatory & quality milestones Ensure research $$$ well spent and align for exit or licensing with the right partner	• Visibility of clear, achievable plan • Early discussion with multinational partner to confirm regulatory strategy	
Part-time quality role	Experience and relevant working knowledge of product development and GMPs in a R&D setting to support pre-market approval	• Relevant guidance documents • Relevant standards • GMPs • Protection of Human Subjects • Institutional Review Boards • Good Laboratory Practices • Good Clinical Practices	• GMP knowledge and experience in the product technology and environment

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START-UP, LATE-STAGE – PRE-APPROVAL			
Founder (technical)	Clear regulatory strategy ahead Clear milestones identified to clear regulatory hurdles	• Same as above for early-stage • Confidence that planned studies will achieve regulatory approval if scientific success, or achieve results that a multinational can utilise in a regulatory application • Stage-gate model	• Review/revise regulatory strategy • Establish if issues of performance, scale and reproducibility exist for the technology
Medsafe website listed consultants, Tech Transfer Office (and/or CEO)	Sufficient budget allocation to support product development activities Adequate regulatory skills and experience, e.g. timeframes, identifying critical data required to support market access Working knowledge of regulatory process Working knowledge of relevant regulations, guidance documents and standards	• Discuss with potential licensee or industry expert	• Licensees have rigid expectations about body of evidence that needs to be generated to demonstrate product potential
CEO	Regulatory impact to R&D, manufacturing and quality Facility licensing Regulatory impacts of scale up	• Identify changes necessary for manufacture of investigational product for clinical trial purposes • GMP facility registration+ • Identify changes required for manufacture at scale	
Investor/ Commercial partner	Regulatory capability	• Regulatory risk (milestones achievable): • Science/engineering • Standards • Timeframes	• Is regulatory advice/support budgeted for pre-clinical, clinical, pre-market? • Is regulatory pathway clearly understood and resourced for successful execution? • Budget ~$1.8m to get a therapeutic to a point where a clinical trial can be initiated • Inputs to valuation according to risk weighted NPV

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START-UP, LATE-STAGE – PRE-APPROVAL			
Quality manager	Experience and relevant working knowledge of product development and GMPs in a R&D setting to support pre- and post-market approval Facility compliance	• Relevant guidance documents • Relevant standards • GMPs • Protection of Human Subjects • Institutional Review Boards • Good Laboratory Practices • Good Clinical Practices	• GMP knowledge and experience in the product technology • Qualified Person • Product Release
Regulatory manager	Experience and relevant working knowledge of regulatory process, product development, GMP and commercialisation to support pre- and post-market approval Ongoing monitoring for regulatory updates Experience and relevant working knowledge of regulatory process, product development, GMP and commercialisation to support pre- and post-market approval	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Labelling • Protection of Human Subjects • Institutional Review Boards • Establishment and Listing • Good Laboratory Practices • Good Clinical Practices • Post-market approval requirements • Corrections & Removals • Pharmacovigilance	• Regulatory knowledge and experience in the product technology • Knowledge and experience of regulatory process for the region of interest • Ability to deliver a regulatory strategy that is current for the product and regulatory environment which is relevant to the business • Knowledge and experience in regulatory inspection/ assessment • Ability to integrate regulatory knowledge into business strategy • Knowledge and experience in reimbursement process
Clinical Manager	Relevant working knowledge of clinical research and operations with respect to the conduct of regulatory process and environment in product development to support pre- and post-market approval Clinical expertise in the technology space/clinical application space	• Relevant guidance documents • Relevant standards • Protection of Human Subjects • Institutional Review Boards • IND • NDA/BLA • Product development • Lifecycle Management • Good Clinical Practices	• See Clinical Trial touchpoint map • Product Development

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START-UP, LATE-STAGE – PRE-APPROVAL			
R&D manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Regulatory expertise in the technology space/clinical application space	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMPs • Formulation development • Good Laboratory Practices	• Strong scientific skills for the product technology under development • Skills in tech transfer, R&D, formulation development, test method development and manufacturing • Knowledge and experience in product development
Manufacturing manager	Relevant working knowledge of regulatory process and environment in product development, tech transfer and commercialisation to support pre- and post-market approval Manufacturing expertise in the technology space	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Process Controls • Good Laboratory Practices	• Knowledge and experience in manufacturing standards for the environment the technology will be developed & manufactured • Skills in R&D, test method development, process development, tech transfer and manufacturing • Knowledge and experience in Process Controls, Validation etc.
Marketing/Sales manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Marketing and Sales expertise in the technology / clinical application space	• Labelling • Product development • Pharmacovigilance • Good Clinical Practices	• Knowledge and expertise in establishing label claims • Knowledge and expertise in requirements needed to commercialise pharma/biotech products • Knowledge in market, specifically of competitors for the sector

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START-UP, LATE-STAGE, POST-APPROVAL			
Founder (technical)	Clear milestones identified to meet approval requirements including but not limited to clinical studies, as applicable	• Same as above for late-stage pre-approval	• Modifications to new product or changes to approved products • Post-market feedback loop into next-gen product • Confidence that planned studies will achieve post-market requirements, if applicable • Or achieve results that multinational can utilise in a different regional dossier • Ensure that the existing product remains safe and effective once it's on the market
Medsafe website listed consultants, Tech Transfer Office (and/or CEO)	Adequate budget and skills for regulatory support	• Discuss with potential licensee or industry expert	• Licensees have rigid expectations about type of studies that demonstrate product potential
Investor	Regulatory capability	• Is reg advice/support budgeted for? • Pre-clinical • Clinical • Post-market	• Ensure that the existing product remains safe and effective once it's on the market. • Manage/mitigate product risk • Manage/mitigate patient risk
	Regulatory risk (milestones achievable): • Science • Standards • Timeframes	• Inputs to valuation according to risk weighted NPV	
Quality manager	Experience and relevant working knowledge of product development and GMPs in a R&D setting to support pre- and post-market approval. Facility compliance	• Relevant guidance documents • Relevant standards • GMPs • Protection of Human Subjects • Institutional Review Boards • Good Laboratory Practices • Good Clinical Practices	• GMP knowledge and experience in the product technology • Qualified Person • Product Release

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START-UP, LATE-STAGE, POST-APPROVAL			
Regulatory manager	Experience and relevant working knowledge of regulatory process, product development, GMP and commercialisation to support pre- and post-market approval	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Labelling • Protection of Human Subjects • Institutional Review Boards • Establishment and Listing • Good Laboratory Practices • Good Clinical Practices • Post-market approval requirements • Corrections & Removals • Pharmacovigilance	• Regulatory knowledge and experience in the product technology • Knowledge and experience of regulatory process for the region of interest • Ability to deliver a regulatory strategy that is current for the product and regulatory environment which is relevant to the business • Knowledge and experience in regulatory inspection/assessment • Ability to integrate regulatory knowledge into business strategy • Knowledge and experience in reimbursement process
	Ongoing monitoring for regulatory updates Experience and relevant working knowledge of regulatory process, product development, GMP and commercialisation to support pre- and post-market approval		
Clinical manager	Relevant working knowledge of clinical research and operations with respect to the conduct of regulatory process and environment in product development to support pre- and post-market approval Clinical expertise in the technology space/clinical application space	• Relevant guidance documents • Relevant standards • Protection of Human Subjects • Institutional Review Boards • IND • NDA / BLA • Product Development • Lifecycle Management • Good Clinical Practices	• See Clinical Trial touchpoint map • Product development

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START-UP, LATE-STAGE, POST-APPROVAL			
R&D manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Regulatory expertise in the technology space/clinical application space	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMPs • Formulation development • Good Laboratory Practices	• Strong scientific skills for the product technology under development • Skills in tech transfer, R&D, formulation development, test method development and manufacturing • Knowledge and experience in product development
Manufacturing manager	Relevant working knowledge of regulatory process and environment in product development, tech transfer and commercialisation to support pre- and post-market approval Manufacturing expertise in the technology space	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Process Controls • Good Laboratory Practices	• Knowledge and experience in manufacturing standards for the environment the technology will be developed & manufactured • Skills in R&D, test method development, process development, tech transfer and manufacturing • Knowledge and experience in Process Controls, Validation etc.
Marketing/Sales manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Marketing and sales expertise in the technology/clinical application space	• Labelling • Product development • Pharmacovigilance • Good Clinical Practices	• Knowledge and expertise in establishing label claims • Knowledge and expertise in requirements needed to commercialise pharma/biotech products • Knowledge in market, specifically of competitors for the sector

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GROWTH, ESTABLISHED AND EXPANSION			
CEO	Balance of in-market with local support In-house vs consultant Expert vs in-house trained	• Specialised in-market vs special knowledge of product • Budget and timing aspects • In-house training is product specific, expert has broader strategic insight	
CTO (CSO, CMO)	Alignment of Regulatory Affairs with other technical and functional departments	• Drug discovery • Pre-clinical • Clinical • Product development • Good Laboratory Practices • Good Clinical Practices	
CFO	Budget for Reg Affairs mitigates risk of budget blowout on R&D, science/research		
Quality manager	Experience and relevant working knowledge of product development and GMPs in a commercial setting to support pre- and post-market approval Ongoing monitoring of regulatory updates	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • Protection of Human Subjects • Institutional Review Boards • Good Laboratory Practices • Good Clinical Practices • Pharmacovigilance	• GMP knowledge and experience in the product technology, and quality system • Qualified Person

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Regulatory manager	Experience and relevant working knowledge of regulatory process, product development, QSR and commercialisation to support pre- and post-market approval Ongoing monitoring for regulatory updates Regulatory expertise in the technology space/product application space.	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Labelling • Protection of Human Subjects • Institutional Review Boards • Establishment and Listing • Good Laboratory Practices • Good Clinical Practices • Post-market approval requirements • Corrections & Removals • Pharmacovigilance	• Regulatory knowledge and experience in the product technology, as well as facility and product compliance. • Knowledge and experience of regulatory process for the region of interest • Ability to deliver a regulatory strategy that is current for the product and regulatory environment (relevant to the business) • Knowledge and experience in regulatory inspection/ assessment • Ability to integrate regulatory knowledge into business strategy • Knowledge and experience in reimbursement process
Clinical manager (if clinical trials or publications are required)	Relevant working knowledge of compliant clinical research and operations with respect to the conduct of clinical trials to support pre- and post-market approval requirements Clinical expertise in the technology space/clinical application space.	• Relevant guidance documents • Relevant standards • Protection of Human Subjects • Institutional Review Boards • IND • NDA/BLA • Product development • Good Clinical Practices	• See Clinical Trial touchpoint map
Manufacturing manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Regulatory expertise in the technology space	• Relevant guidance documents • Relevant standards • IND • NDA/BLA • GMP • Process Controls • Good Laboratory Practices	• Knowledge and experience in manufacturing standards for the environment the technology will be developed & manufactured • Skills in R&D, test method development, process development, tech transfer and manufacturing • Knowledge and experience in Process Controls, Validation etc.
Marketing and sales manager	Relevant working knowledge of regulatory process and environment in product development and commercialisation to support pre- and post-market approval Expertise in the technology space (nice to have)	• As above	• Knowledge and expertise in establishing label claims • Knowledge and expertise in requirements needed to commercialise pharma/biotech products • Knowledge in market, specifically of competitors for the sector

CTO = Chief Technical Officer; CSO = Chief Science Officer; CMO = Chief Medical Officer

- FDA approval (FDA must respond within 12 months for standard review and 8 months for priority review).
- Registration and listing prior to commercialisation:

Notes for Fast Track and similar approvals:

The FDA has developed four distinct approaches to making such drugs available as rapidly as possible that treat serious diseases especially when the drugs are the first available treatment or if the drug has advantages over existing treatments. They are Priority Review; Breakthrough Therapy; Accelerated Approval; Fast Track.

Some of these pathways are aligned with beneficial IP settings, so where a founder has discretion to choose the clinical indication of a drug that may treat multiple conditions the optimisation of pathway and IP settings should be explored.