

Division of Plasma Protein Therapeutics (DPPT)

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Division Director

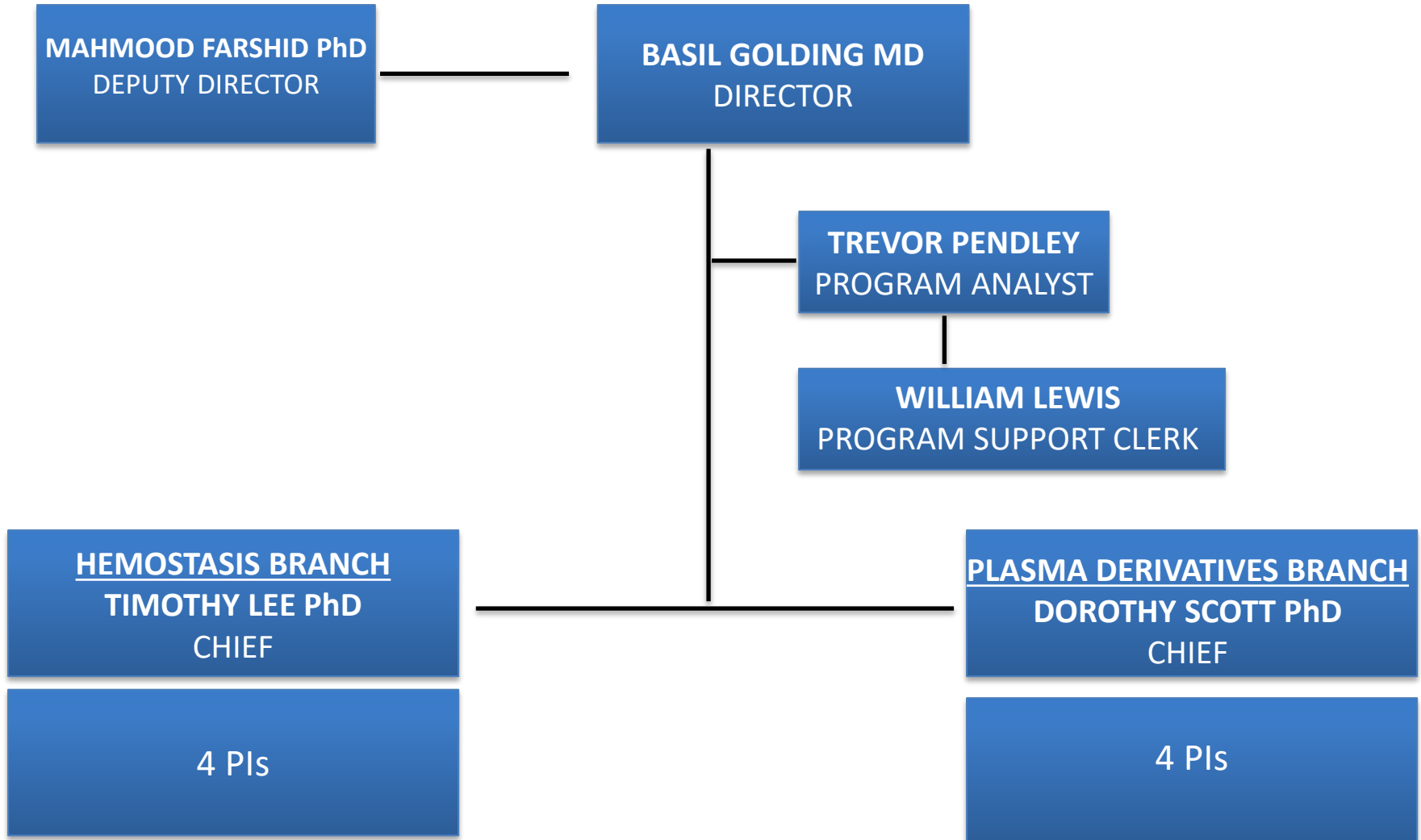
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Site Visit: November 1, 2017

Outline

- Division Organization
- Regulatory responsibilities
- Research

DIVISION OF PLASMA PROTEIN THERAPEUTICS (DPPT)



DPPT Workload (% of time)

	<u>Research</u>	<u>Review</u>
PIs	50	50
Staff Fellows	50	50
ORISE fellows	100	0
Chemists	50	50

DPPT: Regulatory Function

- Applications and products
 - Biologics (diverse complex proteins) and devices
- Evaluation process of products
 - scientific review of manufacturing processes
 - laboratory investigation if needed
 - assay development
 - standards are developed for lot release of different products and are often distributed as CBER and/or international standards
- Surveillance
 - Inspections
 - Adverse Event Reports
 - Product failures
- Legal Framework
 - CFR, PDUFA, MDUFMA, FD&C Act, Guidances

DPPT: Other Tasks

- Policy and Guidance documents
- Advisory Committees
- Harmonization: ICH guidance, EMA, Canadian Health Authority
- Liaisons:
 - Government Agencies (e.g. NIH, CDC)
 - Industry (PPTA)
 - Patient groups (e.g. NHF, IDF)
- Communications
 - Webposting/Letters
- Workshops
- Citizen Petitions

DPPT: Regulatory Process

Decisions - based on scientific data showing safety, efficacy and purity

– decision-making process

- internal review
- presentations to Advisory Committees
- meetings with manufacturers
 - Pre IND/IDE
 - IND/IDE
 - Pre-BLA/Pre-PMA
 - Late Cycle Meeting

DPPT Products - Plasma Derivatives Branch

- **General Immune globulins (IGIV, IGSC, IGIM) for PID, auto-immune disease (e.g. ITP), neurological diseases (e.g. CIDP)**
- **Specific Immune globulins enriched for particular antibody specificities (e.g. rabies, tetanus, botulinum, anthrax and snake bites)**
- **Enzyme inhibitors for hereditary deficiencies (e.g. Alpha-1 Proteinase Inhibitor and C1 Esterase Inhibitor)**

DPPT Products - Hemostasis Branch

- **Coagulation Factors**
 - Factors VIII and IX (Human plasma-derived & Recombinant)
 - Factor VIII/von Willebrand Factor Complex
 - Fibrinogen Concentrate
 - Factor XIII
- **“Bypassing” Products**
 - Anti-Inhibitor Coagulant Complex (e.g., FEIBA)
 - Recombinant activated Factor VII
- **Prothrombin Complex Concentrates (e.g. Kcentra)**
- **Anti-coagulants**
 - Protein C
 - Antithrombin III (Human plasma-derived & Recombinant)
- **Hemostatic Agents**
 - Thrombin (Bovine, Human & Recombinant)
 - Fibrin Sealant
 - CryoSeal FS System
 - Fibrin Sealant Patch

Scope of DPPT Research

- Efficacy of Immune Globulin Products (Flu, Ebola, Zika and HCV)
- Study of the regulation of blood coagulation by coagulation Factors VIIa, IXa and XIa
- Aggregates in protein products
- Clearance mechanisms of proteins: Factor VIII
- Immunogenicity of protein therapeutics
- Pharmacogenomic and codon optimization studies of proteins
- Materno-fetal transfer of antibodies
- Assay and standard development
- Counter-terrorism and Pandemics (e.g. Influenza)

Publications

76 peer –reviewed papers - last 5 years

Thank You!