

Development and Initiation of the International Multi-centre Paediatric Portal Hypertension Registry (IMPPHR)

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INTRODUCTION: The aetiology, pathophysiology, management and evolution of paediatric portal hypertension (PHT) is distinct from that which occurs in adults. As such application of adult-derived evidenced based approaches to children with liver disease or portal vein thrombosis may be problematic. Unfortunately, there is very limited high-quality data from which to derive guidelines to the management of PHT in children. IMPPHR was developed to derive large-scale international data that would enhance our knowledge of PHT.

METHODS: In December 2018, in follow up of the Baveno VI Paediatric Satellite Symposium (Hepatology 2016;63:1368-1380) an executive committee was established to develop IMPPHR.

There are 3 major foci of data collection in IMPPHR

- morbidity and mortality of first variceal haemorrhage
- feasibility of primary prophylaxis of oesophageal varices
- approaches to 2ndary prophylaxis of variceal haemorrhage

The principles of the registry development included

- ascertainment of objective data
- focused data collection on relevant information
- retrospective approach amenable to regulatory agencies
- relevance to addressing the question of the advisability of the use of primary endoscopic variceal prophylaxis.

RESULTS: The IMPPHR executive committee met monthly to quarterly for 2 years and developed a series of case report forms (CRF). Potential subjects in the registry were children who either underwent 1st variceal haemorrhage or primary endoscopic prophylaxis of varices between July 1, 2012 and June 30, 2017 with follow up until June 30, 2020. Each centre provided characteristics and clinical activity accrued between January 1, 2018 and December 31, 2019 to inform resources and approaches available in clinical practice at that centre.

Major CRF include: 1) Subject demographics, 2a) First variceal haemorrhage and secondary prophylaxis data, 2b) Primary endoscopic variceal prophylaxis data and 3) Final outcome data.

The multi-centre database is available for on-line data entry behind the firewall at Baylor College of Medicine and was IRB approved at Baylor College of Medicine on June 30, 2020. Negotiation of data use and material transfer agreements was commenced after this IRB approval and data entry is underway. As of June 7, 2022 appropriate database design and function has been confirmed by entry of 83 subjects from the executive committee member's clinical sites. The IMPPHR sites for the executive committee encompass geographic areas with a cumulative population of ~ 50M; 1792 paediatric hospital beds and 408 paediatric intensive care beds. PHT related clinical activity amongst the involved centres is summarized in Table 1 (Portal Hypertension Related Clinical Activity Amongst Involved Centres). Data entry electronic forms are shown in the figures below (1-3).

Clinical Activity (# 1.1.18-12.31.19)	Mean	SD	Range	Cumulative
Liver Transplants	51.3	32	7 - 102	359
Living Donor Liver Transplant	8.0	9.8	0 - 26	56
TIPS	1.1	1.9	0 - 5	8
Portosystemic Shunt	2.1	3.5	0 - 10	15
MesoRex Bypass	3.1	3.4	0 - 8	22
Variceal band ligation (VBL)	56.7	70.0	7-200	397
# Individuals performing VBL:# /individual	5.0:14.4	2.9:18.0	2-9:2.3-49.5	
# Individuals performing EST:# /individual	3.0:9.3	1.8:11.3	2-7:1.0-32.5	

Fig 1. Demographic data form (above)
Fig 2. Primary endoscopic prophylaxis form (below left)
Fig 3. Final status form (below right)

CONCLUSION: A multi-centre registry focused on paediatric PHT, particularly management of oesophageal varices, has been developed with ongoing patient data entry. Ultimately, 50-100 centres will contribute a total of > 1000 cases to the registry. This resource will provide much-needed information to guide clinicians caring for children with PHT.

<https://www.texaschildrens.org/international-multi-center-pediatric-portal-hypertension-registry>

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