

Translational medicine: Bedside to bench to bedside- A case study in pediatric ARDS

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Rare diseases are designated as affecting less than 200,000 individuals (US) and of the approximately 7000 designated rare diseases, the majority of these occur in pediatric patients, and across international boundaries. An example is pediatric ARDS (Acute Respiratory Distress Syndrome) that is not diagnosed until a previously healthy child presents in the pICU with severe symptoms and in which more children die each year than from cystic fibrosis and leukemia, combined. The Nathaniel Adamczyk Foundation (NAF) is focused on identifying risk factors and opportunities for prevention of this devastating disease. Both the diagnosis and patient management are challenged by having to deal with a syndrome in a critical care situation in a heterogeneous patient population.

NAF has undertaken the development of an (inter) national tissue and data repository to support both clinical research and enhanced clinical decision support for patient management. Creation of an analytical platform to integrate, access and analyze temporal clinical data ranging from the pICU to also incorporate neo-natal ICU and pregnancy history is underway with a prototype already in testing. Analytical methods are being evaluated in collaboration with Dr. Mike Quasney (University of Michigan), the Virtual Pediatric ICU (VPS) and PALISI (Pediatric Acute Lung Injury and Sepsis Investigators). This effort is exploring expanded international partnerships in both Europe and China to increase the accessible data for analysis and to further participate in the development of better diagnostic standards.

We will present the unique translational approach adopted by NAF and developed by CNR and IPQ to identify and address the clinical problem in the pICU for patient management and how this is based on disease and patient stratification. The validation of this approach with clinicians then enables basic researchers to begin to identify early risk factors and/or biomarkers in asymptomatic patients.

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