

[NM-258] Anesthetic Considerations and Complications during Phase I/II of Adeno-Associated Virus Mediated Alpha-Glucosidase Gene Therapy to the Diaphragm

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Infantile Glycogen Storage Disease type II (GSD-II) or Pompe's disease presents within the first months of life. It is characterized by a deficiency in the enzyme acid alpha-glucosidase (GAA). Absence of GAA leads to the accumulation of glycogen in all cells but most significantly striated muscle and motorneurons. Until recently, it was fatal within the first two years of life from cardiomyopathy and respiratory complications. With the development of GAA enzyme replacement therapy (ERT), these patients are experiencing increased life-expectancy with improvement in cardiac function and muscle tone. Unfortunately, there is no concurrent improvement in long-term respiratory function. Many of these ERT patients are now requiring invasive ventilator support.

General anesthesia and bilateral thoracoscopies were performed on five subjects enrolled in a phase I/II clinical trial of AAV-mediated GAA gene therapy. All had tracheostomies and required chronic mechanical ventilation at baseline. Anesthesia was induced with a combination of low-dose sevoflurane, ketamine and versed. An arterial line for hemodynamic monitoring was placed. Careful respiratory parameters in regards to ventilation and oxygenation were maintained during the bilateral thoracoscopy. Blood pressures were maintained to ensure adequate coronary perfusion due to a thickened myocardium. All five patients were taken post-operatively to the intensive care unit for postoperative recovery. There were no anesthetic complications noted in the perioperative period. One patient experienced bleeding from inadvertent lung puncture during port placement, but did not require a blood transfusion. This patient did require chest tube placement.

There are case reports and series of cardiac and respiratory complications in patients with Pompe's disease undergoing general anesthesia. With the current diagnosis and treatment recommendations of receiving ERT soon after diagnosis, many more patients may undergo a general anesthesia. These five patients illustrate the potential complications and intricacies that must be managed while operating in the thorax. Most perioperative complications occur in the cardiopulmonary system and an updated knowledge of changes in therapy must be maintained to care for these dynamic patients.

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