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# The use of port-a-caths in adult patients with Lysosomal Storage Disorders receiving Enzyme Replacement Therapy-one centre experience

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ABSTRACT

Port-a-cath is a widely used device in patients with long-term venous access demand such as frequent or continuous administration of medications such as Enzyme Replacement Therapy (ERT), chemotherapy delivery, blood transfusions, blood products, and fluids. Patients with Lysosomal Storage Diseases (LSDs) often require recurrent courses of ERT. We reviewed our experience of using port-a-caths in patients with LSDs with the focus on challenges and complications associated with these catheters. Among 245 adult patients who were treated with ERT, twenty patients (8.2%) had a port-a-cath inserted due to poor venous access. Six patients were using their first port whereas five other patients had their port-a-caths replaced at least once. The remaining six patients had inactive port-a-caths. The majority of patients with active port-a-caths never missed more than one consecutive infusion, although one patient missed 2 consecutive infusions whilst on holiday. We identified significant gaps in patients' and their families' understanding of the management of port-a-caths and risks associated with them. It resulted in producing a leaflet and designing an educational program for our LSD patients.

## 1. Introduction

A port-a-cath (also called Totally Implantable Vascular Access Device, TIVAD) is a widely used device in patients with long-term venous access demand [1,2] such as frequent or continuous administration of medications such as Enzyme Replacement Therapy (ERT), chemotherapy delivery, blood transfusions, blood products, nutrition and fluids [2]. The device consists of a port and an intravascular catheter which is placed into a large vein in the upper chest area and appears as a bump under the skin [2]. Due to the direct access into the superior vena cava, chemotherapy or ERT can be safely administered through the port. The device can also be used to draw blood for testing [2].

The use of a port-a-cath allows reliable venous access to patients with poor venous access due to abnormal skin, previous repetitive puncturing or underlying condition causing poor circulation or abnormal vasculature. It helps avoid the puncture wounds and damage to the blood vessel that would result from repeated peripheral access to veins. A port-a-cath insertion is usually carried out under sedation or general anesthetic and a gripper needle is used to gain access. Gripper needles can also be kept in for up to seven days if there are no signs of infections if access is required more frequently. Importantly, the device can be regarded as semi- permanent and used as long as it is needed (on average 2–6 years) [3] or it can be removed when no longer needed.

Patients requiring central venous access devices are generally susceptible to complications and disability because of their underlying health condition. This vulnerability is worsened by the risk of adverse events associated with the insertion and management of these devices [4]. Potential documented risks of port-a-cath include bleeding or bruising, occlusion or blockage of line due to clot, dislodgment of port or line, damage to the port-a-cath [2,4–10] and migration of the port-a-cath to the duodenum [11]. Port-a-caths require less maintenance, routine cleaning or dressing, when compared to other indwelling catheters, but will require a heparin flushing when not in use for more than 4 weeks [1,2]. Due to the port-a-cath's design, there is a very low infection risk when compared with other indwelling lines such as the Hickman line. Infections may still be a risk in the case of immunosuppressed patients [2].

Lysosomal Storage Diseases (LSDs) are a heterogeneous group of conditions characterized by an abnormal build-up of storage materials in lysosomes as a result of enzyme deficiencies. The clinical presentation is multi-systemic. LSDs affect different parts of the body, including the musculoskeletal system, central nervous system, skin, heart and kidneys [12–19]. Patients affected by these conditions often require recurrent courses of Enzyme Replacement Therapy (ERT) [12]. ERTs

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are now available for several LSDs such as Gaucher disease [13], Pompe disease [14], Fabry disease [15], MPS I [16], MPS II [17], MPS IV [18], MPS VI [19] and potentially for MPS VII soon. The key to success of the therapy is an early onset of treatment. As the venous access in children is traumatic and challenging in most cases, to achieve maximum benefit of treatment and improve the compliance, TIVADs are frequently inserted. These devices have been used extensively in children with cystic fibrosis and in children suffering from malignancies. In many of these cases life expectancy was estimated as short, therefore the potential long-term risks of venous catheters were rarely considered. Over time these patients may lose peripheral access, and indwelling venous catheters are required. There are currently no available guidelines on the management of port-a-caths in patients with Inherited Metabolic Disorders. Furthermore, port-a-caths are not frequently used in adult patients with these conditions and there is also no clear policy in place for the current homecare companies in the UK that are managing these devices in patients on ERT.

The objective of this project was to develop a concise leaflet containing guidelines for our adult patients with LSDs, to educate them about the risks and benefits of the procedure prior to placement of these devices. This article outlines our experience in a large adult center of using port-a-caths in patients with LSDs with the focus on challenges and the complications associated with them.

## 2. Methods

### 2.1. Patients

We reviewed patients with Inherited Metabolic Disorders such as Mucopolysaccharidosis (MPS), Fabry diseases, Pompe disease, Gaucher disease attending our Adult Inherited Metabolic Disorders clinics at the Salford Royal NHS Foundation Trust (SRFT). Demographic information was obtained from our Electronic Patient Record and from the Transition Clinic documentation.

### 2.2. Port-a-caths

Details regarding the type of catheter, duration of its use, type and number of complications and the use of anticoagulant medication were extracted. We explored the number of port-a-caths each patient had and the length of time they were used. We also reviewed any missed home infusion reports for the patients who have active ports.

## 3. Results

Among 245 adult patients who were treated with ERT, 20 patients (8.2%) had a port-a-cath inserted due to poor venous access. Of the 20 patients (mean age 33 years (20–69)), twelve were known to have an MPS disorder, five patients had Fabry disease, one patient had Gaucher disease and two patients had Pompe disease. Ten of the patients had their port-a-cath inserted in childhood and ten other adults had ports inserted as adults due to deteriorating venous access; one of whom had the port-a-cath inserted under pediatric care because the Adult Inherited Metabolic Unit in Salford did not exist at the time. Importantly, in most cases the management of the port-a-cath was not communicated during the transition from Children's Hospital to the Adult Inherited Metabolic Medicine Unit and there was no supporting documentation available. Of the 20 patients, six were on their first port, five were using their second port and three patients were unsure how many devices they had throughout their ERT period, including the date of their insertion or placement. The remaining six patients had inactive port-a-caths *in situ*. The majority of patients with active port-a-caths never missed more than one consecutive infusion, although one patient missed two consecutive infusions whilst on holiday. Additionally there were three patients who had port-a-caths in place but they have now been removed in adulthood as they were no longer needed and venous access was achieved *via* repeated cannulation. The average length of time the port-a-cath was in place was 7 years (0.6–14).

The main challenges associated with using port-a-cath in patients with LSDs were their chest deformities and extreme short stature in MPS disorders. Complex airway, short thick neck, limited cervical spine movement and the risk of cardiovascular complications requires specialist assessment and anesthetist care before the port-a-cath insertion. These are the primary determining factors that were taken into consideration when balancing benefits against the risks of this procedure in our patients with MPSs. In addition, in MPSs and Fabry disease patients who suffer from arrhythmias, thrombus formation, as a complication of using port-a-caths, may enhance their risk of cardiovascular event.

Therapies for LSDs are life-long compared to patients suffering from malignancies which are usually exposed to shorter and more intensive courses of chemotherapy. Patients with LSDs are infused with proteins which have different characteristics compared to antibiotics, used for treating infections in patients affected by cystic fibrosis, and chemotherapy compounds. The risks of infusion-associated reactions are high in LSD patients whereas the role of antibodies is not even considered as a problem in conditions other than LSDs.

The main complications associated with using the venous catheter were infection and allergic reaction in two patients and blood thrombus in one case. In two patients the port-a-cath was pushed through the skin. In another case port-a-cath ruptured and was removed as an emergency.

We identified significant inconsistencies in patients' and their families' understanding of the management of port-a-caths and the associated risks. Their understanding of port-a-cath-related benefits and risks was limited in most cases with the lack of formal training on the maintenance of this device being the most likely cause. Few patients stopped using their port-a-cath and as a result they forgot to mention to their family members or physicians that they still had a foreign body *in situ*. Due to poor venous access some patients and their families demanded to have a permanent catheter inserted. They were not fully aware that the procedure can be challenging, requires genetic anesthetic sedation, pre-operative assessment and ideally should be performed in a center of expertise.

## 4. Discussion

In view of the absence of any published data on using port-a-caths in patients with LSDs requiring frequent infusions, this article outlines our experience from one of the largest LSD centers. We currently infuse the following numbers of patients: 154 Fabry disease, 27 with Gaucher disease, 12 with MPS I, 12 with MPS II, 6 with MPS IV, 32 with Pompe and 2 with Cholesterol Esterase Storage Disease. There is clearly an increasing demand for the use of devices to facilitate regular infusions among the adult patients treated with ERT. Our retrospective audit showed that complications are relatively rare among adult patients despite their long-term use and missing transition documents from the pediatric care setting.

A port-a-cath is often the first choice of vascular access device in adults because of the reduced risk of an infection, and the reduced need for flushing with heparin solution in between infusions [20–23]. From our experience, patients awaiting port-a-cath insertion often have a temporary line inserted to avoid any delay in starting their ERT; however, the temporary line can break causing delays in treatment. Although patients with a metabolic condition only require ERT infusion once a week or once a fortnight, those using a temporary line alternate weeks also require the line to be flushed every week. This incurs an extra cost to metabolic services and is associated with additional training for local community nurses.

### 4.1. Inactive/unused port-a-caths

There is some discrepancy as to how frequently a port-a-cath should