



British Paediatric Surveillance Unit

## PUBLIC INFORMATION LEAFLET

### END-STAGE RENAL DISEASE IN EARLY INFANCY

**What is End-stage renal disease in early infancy?** Each year around 20-30 children in the UK and Ireland are born who develop irreversible Kidney failure sometime in the first few months of life. This is called End stage renal disease in early infancy (ESRDi). There are a number of causes (see below) but the end result is the same. The kidneys do not work properly and are unable to manage the fluids, salts and minerals that the body uses, or remove waste products from the blood-stream. As a result, infants with this problem become unwell with poor growth and need specialised treatment to stop them becoming very seriously ill.

**What does the study hope to do?** We want to identify all infants with this problem in the UK & Ireland. We want to look at the problems that these infants face and see how they are treated. We hope this will help us understand the condition better and improve the quality of life for all infants with ESRDi and their families.

**WHAT IS THE BRITISH PAEDIATRIC SURVEILLANCE UNIT (BPSU)?** The aim of the BPSU is to encourage the study of rare conditions in children. It was founded in 1986 by the Royal College of Paediatrics and Child Health, the Health Protection Agency and the Institute of Child Health (London).

**WHAT DOES THE BPSU DO?**

It allows doctors and researchers to find out how many children in the UK and the Republic of Ireland are affected by the particular disease or condition each year – this is called epidemiological surveillance. Doctors can also gather information about all the cases of a particular rare condition so they can begin to understand what might have caused it and how the



## What are the causes of ESRDi?

- *Renal dysplasia.* The kidney tissue does not develop properly before birth and functions very poorly
- *Urine outflow obstruction.* There is a blockage which stops urine draining from the kidney. This then leads to kidney damage. (Posterior urethral valves, where there is an obstruction to urine flow from the bladder is one of the commonest causes in boys)
- *Congenital nephrotic syndrome.* The kidneys leak large amounts of protein. The infant becomes puffy and grows poorly. They often get serious infections.
- *Cystic kidney disease.* Cysts develop in the kidney during growth before birth. These cysts replace normal tissue and the kidneys fail. This disease can be inherited (ie. passed down from generation to generation)

There are other, very rare causes which affect smaller numbers of infants.

## How is ESRDi treated?

Because the kidneys have failed, infants with ESRD cannot tolerate the same type or volume of feeds which other infants can be given. We work with highly skilled dietitians who design specialised diets. This puts the right type of nutrients in the volume allowed for that infant, which helps to improve growth resulting in better overall health. The build-up of waste products seen in ESRD can make the infants sick and weak. Some infants who are well enough, are then, with the agreement of their parents or guardians considered for Dialysis. However, some infants with ESRD can also have other serious medical problems, such as heart disease and problems with development of the brain and nervous system. These infants are often too ill to receive dialysis. They do continue to receive lots of help from medical staff and other hospital professionals to deal with their problems but unfortunately, these infants will not survive their illness.

**HOW DOES THE BPSU WORK?** Each month the unit sends a distinctive orange card to over 2400 consultant paediatricians; the card lists the rare conditions currently being studied. If a doctor has seen a child affected by one of these conditions they tick a box on the card and return it to BPSU.



### **What is kidney dialysis?**

Dialysis is often described as an artificial kidney which can be used to support the infant until they are big enough and well enough to receive a kidney transplant. There are 2 types; Peritoneal and Haemo- dialysis.

Peritoneal dialysis, where a catheter is inserted into the abdomen; this is the first and best choice for dialysing small infants and is usually performed overnight at home, most often 6-7 nights per week.

Haemodialysis is technically very difficult to perform effectively and safely in small babies. A catheter is inserted into a large vein in the chest and positioned at the top of the heart. It is usually reserved for infants larger than 6 kgs and takes place in hospital , 3 times per week.

Dialysis is a very difficult treatment and requires huge commitment from families and hospital staff. There are often complications, such as infection and failure of the catheter to work properly resulting in hospital admissions.. More information of how dialysis works can be found by speaking with a Kidney specialist doctor or nurse.

### **Kidney Transplant**

If the infant is well enough they will be considered for placement on a waiting list to receive a kidney transplant which usually happens when the child reaches a weight of 8-10 kgs. It is now possible for infants to receive a kidney from a living related donor such as a parent or sibling, as well as from a deceased donor. Such surgery in small infants is still very challenging and most children who have had ESRD since infancy do not receive a transplant until they are over 10-12 kgs at least. On some occasions where a living donor is available, a transplant will be arranged to take place before the child needs dialysis, this is called pre-emptive transplantation. Not all children will become well enough to receive a transplant.

#### **WHAT HAS THE BPSU ACHIEVED?**

**PUBLIC HEALTH IMPACT** The BPSU has now helped to undertake surveys of over 100 rare conditions which may affect children. These have helped to increase understanding of why the conditions occur and can help to provide better diagnoses and treatments.



### **The ESRDi study**

At present, there is a lack of information on how common this problem is, or on the difficulties faced by this very important group of patients. More information is needed on the types of treatment used and on how these treatments affect the infants' health. This study has been designed to collect information about all infants in the UK and Ireland who have ESRDi up to the age of 6 months and will look closely at each infant to see what problems they face, look at how they are treated and examine their progress.

**What will the study mean for the infant and their family?** We will not ask anything of the infant or family/guardian. The medical doctors caring for the infants will send us information about the development and treatment of the condition. Through this information we hope to increase understanding of its development and improve treatment.

**Where is this study happening?** The study will be taking place in all hospitals across the United Kingdom, Northern Ireland, the Channel Islands, and the Republic of Ireland.

**How long will it go on for?** The study will continue initially for 13 months. It may be extended to gather more information.

**What are the possible risks and benefits?** The care and treatment received by the infants will receive will not change as a result of the information being collected in this study. Information collected will not identify any individual and confidentiality will be maintained at all times. By collecting the information about infants who develop ESRDi, it is hoped to increase understanding of the condition and help improve treatment.

On receiving the card, the BPSU informs the investigation team, who send the reporting doctor a short confidential questionnaire for more information about the affected child. BPSU researchers never contact families or children and surveillance studies don't affect a child's treatment. The purpose is ONLY to collect information to learn more about the condition.

(From the BPSU Public Information Leaflet – 'Investigating rare childhood conditions for the future health of the nation')

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