

Primary Hyperoxaluria Registry

Date of Summary: April 2010

Patients Enrolled: 232

PH Type

Type	Number	Percent
PH Type 1	190	82
PH Type 2	20	9
Non PH1/PH2	11	5
Unknown	11	5

Of the 232 patients in the registry as of April 2010:

- 190 patients (82%) have Primary Hyperoxaluria Type I
- 20 patients (9%) have Primary Hyperoxaluria Type II
- 11 patients (7%) have Primary Hyperoxaluria of an unknown type
 - There are at least 2 forms of Primary Hyperoxaluria.
- Type I is caused by a deficiency of the enzyme Alanine Glyoxylate Transferase (AGT) which is found only in the liver.
- Type II is caused by a deficiency of the enzyme Glyoxylate Reductase/Hydroxypyruvate Reductase (GR/HPR) found in the liver and other tissues.

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Country

Country	Number	Percent
ALGERIA	1	0
ARGENTINA	1	0
BRAZIL	1	0
CANADA	9	4
CHINA	1	0
COLUMBIA	1	0
DENMARK	1	0
ENGLAND	1	0
GERMANY	13	6
GREECE	2	1
INDIA	7	3
JORDAN	3	1
KAZAKHSTAN	2	1
LEBANON	1	0
MEXICO	2	1
NETHERLANDS	1	0
NEW ZEALAND	1	0
PAKISTAN	3	1
PERU	1	0
POLAND	1	0
SOMALIA	1	0
SOUTH AFRICA	1	0
SPAIN	5	2

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Country

Country	Number	Percent
USA	153	71
VENEZUELA	1	0

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Decade of Diagnosis

Decade	Number	Percent
1960's	9	4
1970's	18	8
1980's	21	9
1990's	50	22
2000's	130	56
2010's	4	2

Of the 232 patients in the registry as of April 2010:

- 9 patients (4%) were diagnosed in the 1960's
- 18 patients (9%) were diagnosed in the 1970's
- 21 patients (9%) were diagnosed in the 1980's
- 50 patients (22%) were diagnosed in the 1990's
- 134 patients (58%) have been diagnosed since the year 2000

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Symptoms and Findings at Presentation

Symptoms and Findings	Percent Present
Urolithiasis	81
Hematuria	38
Abdominal pain	55
Asymptomatic	13
Genetics/family history	83
End stage renal failure	25
Nephrocalcinosis	41

Of the 232 patients in the registry as of April 2010:

- **81% of the patients had a history of urolithiasis (kidney stones)**
- **38% of the patients had a history of hematuria (blood in the urine)**
- **55% of the patients had a history of abdominal pain**
- **13% of the patients were asymptomatic (had no symptoms at all)**
- **83% of the patients had a family history of relatives affected by PH**
- **25% of the patients were already in end stage renal failure**
(End stage renal failure means the patient's kidneys no longer work and they need dialysis or kidney transplantation.)
- **41% of the patients had nephrocalcinosis**
(Nephrocalcinosis is when the kidneys are extensively filled with calcium oxalate crystals that can often cause loss of the kidney.)

Primary Hyperoxaluria Registry

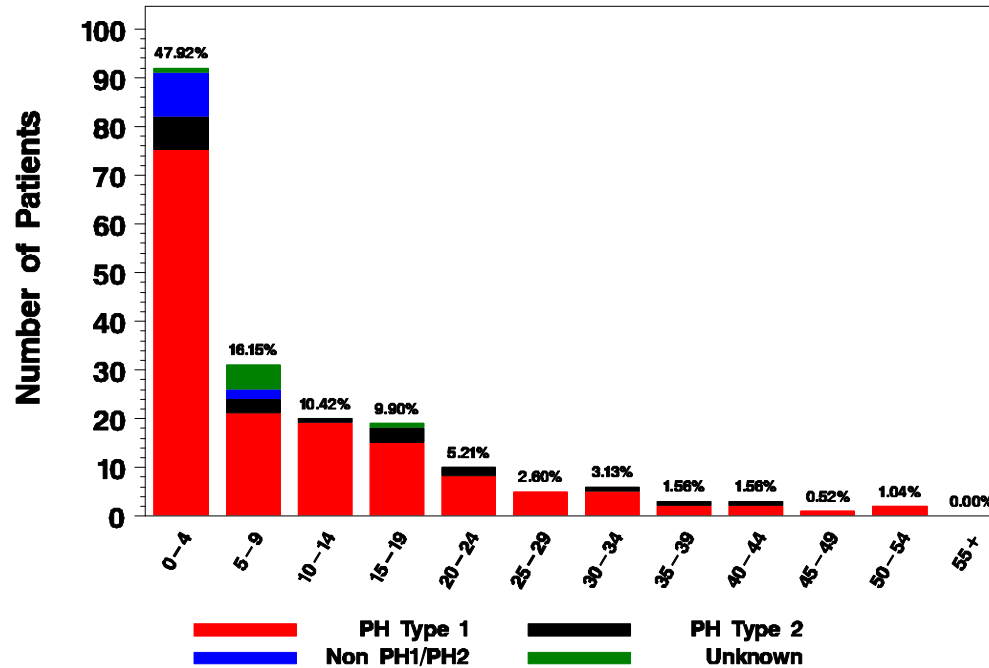
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Status as of Last Follow-up (N=232)

Age in Years at First Symptoms

N=232

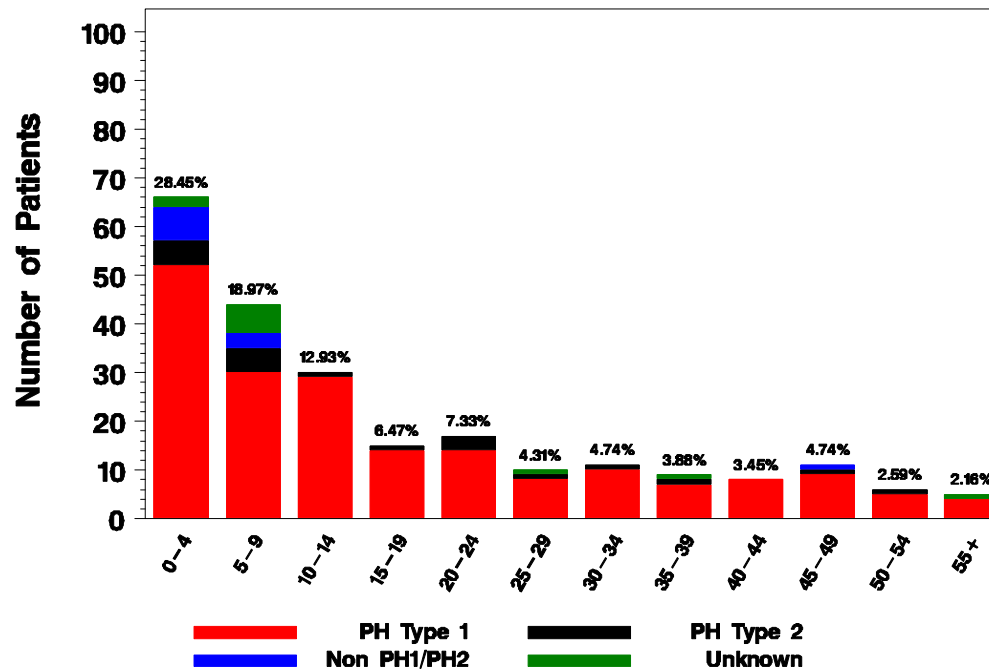


Of the 232 patients in the registry as of April 2010:

- More than 90 patients had their first symptom between the ages of 0-4
- More than 30 patients had their first symptom between the ages of 5-9
- Approximately 20 patients had their first symptom between the ages of 10-19
- Most patients have symptoms before the age of 25
- Type of PH is indicated by color

Age in Years at Diagnosis

N=232

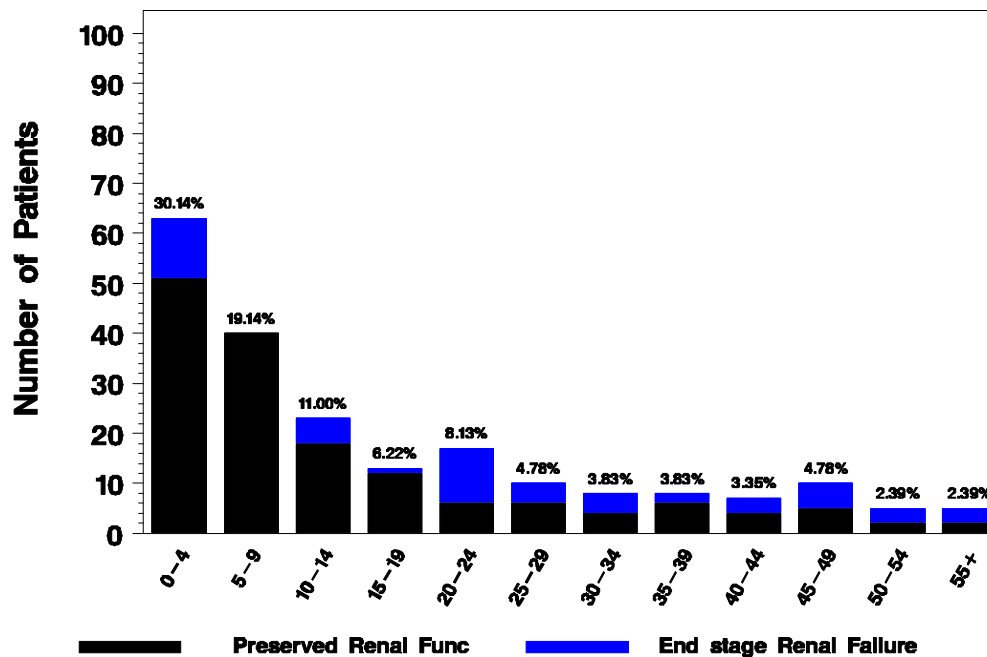


Of the 232 patients in the registry as of April 2010:

- 67 patients were diagnosed between the ages of 0-4
- 46 patients were diagnosed between the ages of 5-9
- The chart shows that most cases of PH are diagnosed before the age of 50 with a large percentage of diagnoses made before a patient's 25th birthday
- Many patients are diagnosed before age 10
- Type of PH is indicated by color

Renal Status by age at Diagnosis

N=232



Of the 232 patients in the registry as of April 2010:

- The majority of patients have functioning kidneys at the time of diagnosis
- However, if a patient is not diagnosed until after age 25, they are more likely to have kidney failure at the time of diagnosis.

Clinical and Lab Findings at Diagnosis

Clinical and Lab Findings at Diagnosis

