

Primary Hyperoxaluria Registry
Date of Summary: April 2009
Patients Enrolled: 203
PH Type

| Type | Number | Percent |
|-------------|--------|---------|
| PH Type 1 | 172 | 85 |
| PH Type 2 | 16 | 8 |
| Non PH1/PH2 | 11 | 5 |
| Unknown | 4 | 2 |

Of the 203 patients in the registry as of April 2009:

- **172 patients (85%) have Primary Hyperoxaluria Type I**
- **16 patients (8%) have Primary Hyperoxaluria Type II**
- **15 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 | 1 |
| ARGENTINA | 1 | 1 |
| BRAZIL | 1 | 1 |
| CANADA | 9 | 5 |
| CHINA | 1 | 1 |
| COLUMBIA | 1 | 1 |
| DENMARK | 1 | 1 |
| GERMANY | 12 | 6 |
| GREECE | 2 | 1 |
| INDIA | 4 | 2 |
| JORDAN | 3 | 2 |
| KAZAKHSTAN | 2 | 1 |
| LEBANON | 1 | 1 |
| MEXICO | 1 | 1 |
| NETHERLANDS | 1 | 1 |
| PAKISTAN | 1 | 1 |
| POLAND | 1 | 1 |
| SOMALIA | 1 | 1 |
| SOUTH AFRICA | 1 | 1 |
| SPAIN | 5 | 3 |
| USA | 135 | 73 |
| VENEZUELA | 1 | 1 |

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Decade of Diagnosis

| Decade | Number | Percent |
|---------------|---------------|----------------|
| 1960's | 8 | 4 |
| 1970's | 18 | 9 |
| 1980's | 20 | 10 |
| 1990's | 50 | 25 |
| 2000's | 107 | 53 |

Of the 203 patients in the registry as of April 2009:

- **8 patients (4%) were diagnosed in the 1960's**
- **18 patients (9%) were diagnosed in the 1970's**
- **20 patients (10%) were diagnosed in the 1980's**
- **50 patients (25%) were diagnosed in the 1990's**
- **107 patients (53%) have been diagnosed since the year 2000**

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Symptoms and Findings at Presentation

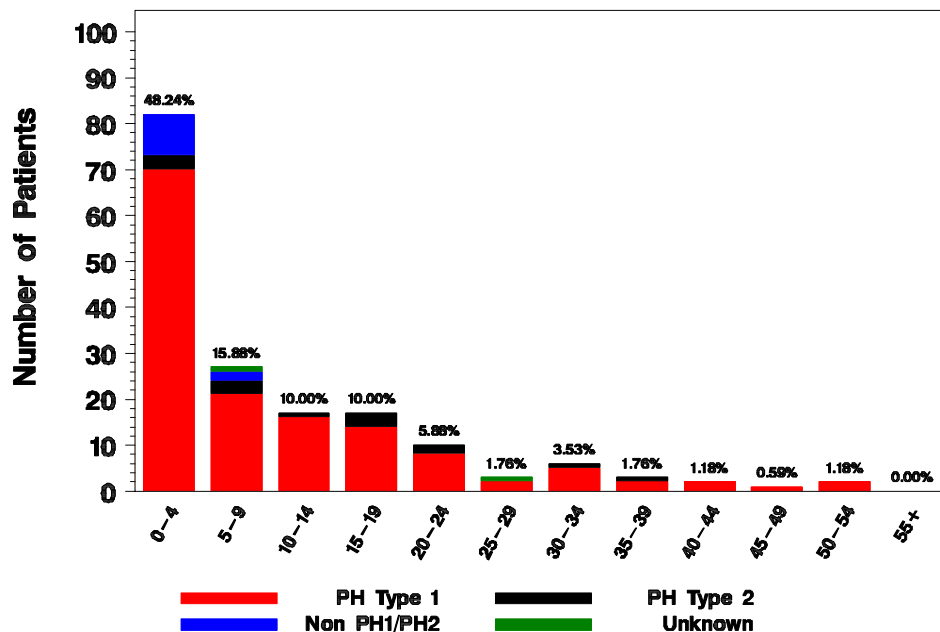
| Symptoms and Findings | Percent Present |
|------------------------------|------------------------|
| Urolithiasis | 84 |
| Hematuria | 36 |
| Abdominal pain | 53 |
| Asymptomatic | 15 |
| Genetics/family history | 80 |
| End stage renal failure | 25 |
| Nephrocalcinosis | 41 |

Of the 203 patients in the registry as of April 2009:

- **84% of the patients had a history of urolithiasis (kidney stones)**
- **36% of the patients had a history of hematuria (blood in the urine)**
- **53% of the patients had a history of abdominal pain**
- **15% of the patients were asymptomatic (had no symptoms at all)**
- **80% 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.)

Age in Years at First Symptoms

N=203

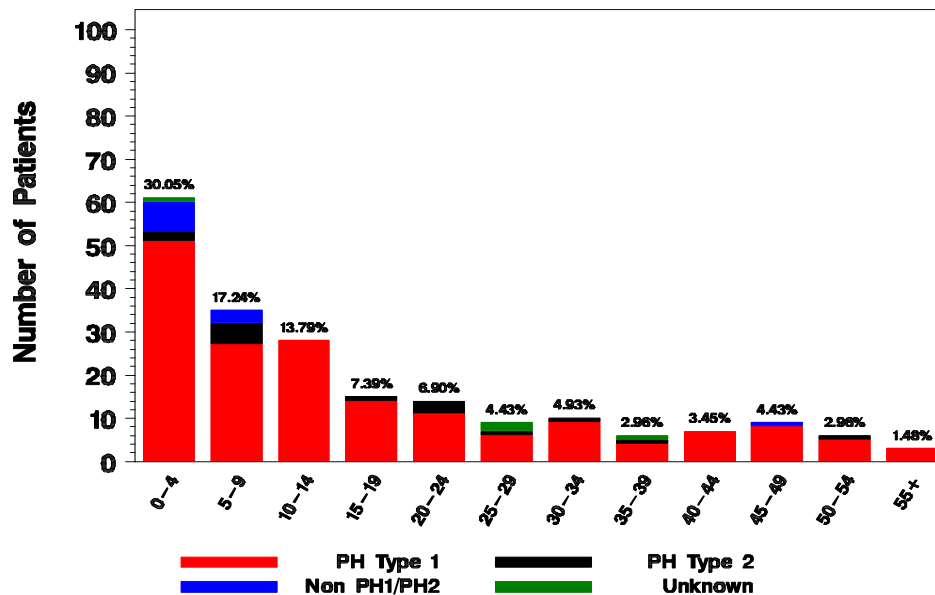


Of the 203 patients in the registry as of April 2009:

- More than 80 patients had their first symptom between the ages of 0-4
- More than 20 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 = 203

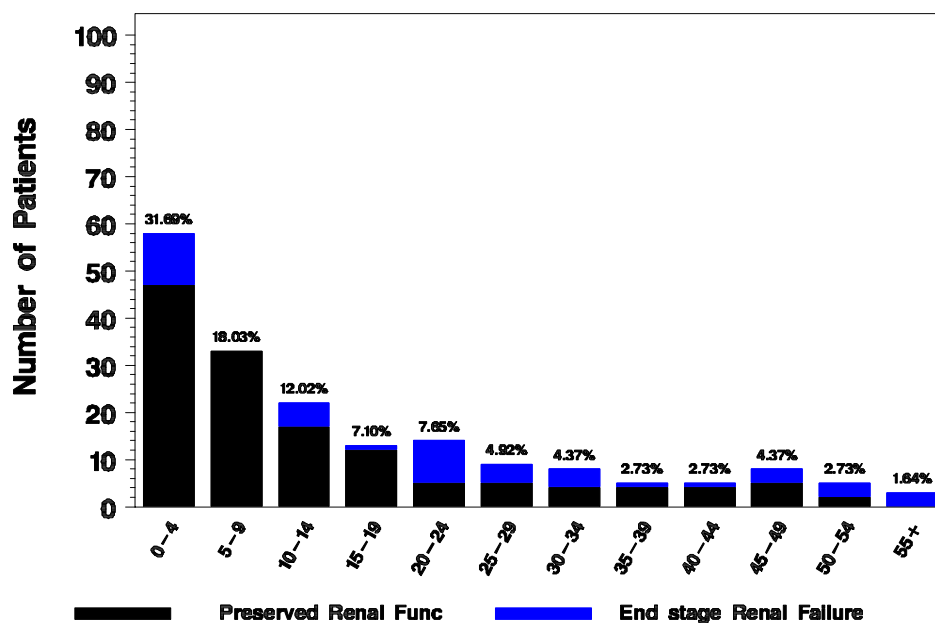


Of the 203 patients in the registry as of April 2009:

- 61 patients were diagnosed between the ages of 0-4
- 35 patients were diagnosed between the ages of 5-9
- 54 patients were diagnosed between the age span of 10-39
- 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 = 203



Of the 203 patients in the registry as of April 2009:

- 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.