

Special Report

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## Cystinosis Patient Day in Iran

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This is a brief report of three cystinosis patient days held by Iranian Society of Pediatric Nephrology (IRSPN) since 2012 with the collaboration of all members from across Iran to collect the latest information about the patients and help to improve the quality of care, increase the awareness of parents and patients about cystinosis, and build a stronger relationship between the victims of this rare disease and the authorities.

**Report**

The first cystinosis patient day and conference for patients with a diagnosis of cystinosis and their parents was held on December 2, 2012 at Ali-asghar Children Hospital. The patient day and conference was organized by Iranian Society of Pediatric Nephrology and sponsored by Orphan-Europe. Thirty-eight families attended this historic first gathering, including 28 children affected by cystinosis. Guest speakers included Dr. Otukesh, Dr. Shakiba, Dr. Akhavan- Sepahi and Dr. Abdizadeh from the Ministry of Health. The speakers provided brief information on the disease and necessary care. The complaints of the parents were heard and discussed by authorities who were representatives of the pharmaceutical companies producing Cystagon and Cystamin –HCl

eye drops. The parents decided to establish an NGO for cystinosis with the support of pediatric nephrologists members of IRSPN ([www.iranspn.com](http://www.iranspn.com)) [1]. (Fig. 1a,b)

The second cystinosis patient day was held on the third day of Iranian Society of Pediatric Nephrology congress on October 23-25, 2013 in Rasool Akram Hospital affiliated with Iran University [2]. There were two parallel sections. The first was a scientific session for physicians and the topics covered the latest information on the genetics of cystinosis, and lungs and endocrine involvement, neurology, nephrology, assessment of height, anemia, and the results of kidney transplantation in Iranian cystinosis patients. The second session was chaired by Dr. Ahmadzadeh from Ahwaz Medical University who presented information on cystinosis in simple words. Almost 50 cystinosis patients attended this seminar with their parents. Dr. Ahmadzadeh had a lecture on cystinosis in simple words for patients and their parents. Pulmonary function test was performed for all patients free of charge.

Dr. Hoseini conducted a question/answer session for patients with the presence of several representatives of Iranian Society of Pediatric Nephrology experienced in cystinosis including Dr. Madani, Dr. Esfehiani, (from Tehran University

of Medical Sciences), Dr. Otukesh (from Iran University of Medical Sciences), Dr. Sharifian (from Shahid Beheshti University of Medical Sciences), and Prof. Niaudet from France (Fig.2 a,b).

Many of the patients were dissatisfied with the availability of Cysteamine eye drop. Cystinosis educational booklets for parents were provided by Orphan Europe Company [3].

The third cystinosis day was held on February 2014 in Nemazee Hospital, Shiraz University of Medical Sciences (SUMS), Shiraz. About 21 families from Fars and surrounding provinces attended this meeting. We invited several expert faculty members to cover most clinical aspects of the disease in order to empower the families to translate what they learn into improved care. In addition, this program was an opportunity for cystinosis patients and their families to become acquainted with one another. (Fig. 3 a, b)

The program had two sessions. In the first session, Dr. Mitra Basiratnia, a pediatric nephrologist from SUMS, made an introductory lecture on cystinosis and the various issues faced by the patients. Then, the genetic aspects of the disease, the importance of transmission, genetic counseling, and prenatal diagnosis were discussed by Dr. Fardaei, a human geneticist from SUMS, in a very simple language.

Since the optimal care of the patients with cystinosis requires a multi-professional approach, the second session was held as a panel discussion composed of several pediatric subspecialties and available data on the number of cystinosis patients in Iran, renal function, pulmonary, neurology and gastrointestinal manifestations, genetic evaluation of the patients, blood abnormalities before renal failure, thyroid function, growth velocity and response to growth hormone, and the results of renal transplantation in Iranian cystinosis patients was presented and discussed [4-13]. During this session, the physicians answered questions in their area of expertise. Moreover, it was a perfect opportunity for the families to discuss their problem with the disease, and preparation and cost of the drugs.

At the end, the following decisions were made:

Appointing a representative for cystinotic families  
Regular, free-of-charge ophthalmologic examination by pediatric ophthalmologists

Readjustment of cysteamine dosage

Negotiation with Sina Daru to solve the shortage of cysteamine eye drop

Negotiation with Rare Disease Foundation of Shiraz for providing therapeutic and pharmaceutical support for cystinotic patients.

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## Cystinosis Patient Day in Iran – Basiratnia M



a



b

**Figure 1. The Family session**



a



b

**Figure 2. Educational session**



a



b

**Figure 3. Shiraz Patient's Day**