

# Spotlight on Rare Diseases

## What is a Rare Disease?

In Europe, a disease is considered rare if it affects fewer than five in 10,000 people (0.05%).<sup>1</sup>



According to EURORDIS (The European Organisation for Rare Diseases), there are more than 6,000 different rare, or “orphan,” diseases.<sup>2</sup> Combined, these diseases affect approximately 30 million European Union citizens.<sup>2</sup>



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AFFECT  
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## Living with Rare Diseases

PEOPLE AFFECTED BY RARE DISEASES MAY FACE CHALLENGES SUCH AS:<sup>3,4</sup>



Lack of information



Difficulty in obtaining a diagnosis, or misdiagnosis



Psychological burden



Few physicians experienced in treating rare disease



Limited support services for the patient and family

Patients and their families may feel isolated and frustrated, and can be emotionally and financially devastated.<sup>4</sup>

## Treatment

**Drug development can be challenging for rare diseases.** Because of the scarcity of patients, pharmaceutical companies can find it **difficult to enroll the number of patients needed** for a rigorous clinical trial. In addition, the scientific knowledge about and **experience with rare diseases is limited.**<sup>5</sup>



These are two of the reasons that the vast majority of rare diseases have no approved treatments.<sup>5</sup>



Medicines intended for treatment, diagnosis or prevention of life-threatening or chronically debilitating rare diseases and disorders are referred to as “**orphan drugs**.”<sup>6,7</sup>

## Janssen's Work in Rare Diseases



Janssen is committed to developing compounds in areas of unmet medical need – and this can include rare diseases. Our work in rare diseases includes:



**Multicentric Castleman's disease**, a disorder in which lymph nodes enlarge because lymphocytes (a type of white blood cells) are over-produced<sup>8,9</sup>



**Mantle cell lymphoma**, a life-threatening and aggressive blood cancer<sup>10</sup>



**Acute myeloid leukemia**, a cancer in the bone marrow and blood occurring when the white blood cell count increases rapidly<sup>11</sup>

**Myelodysplastic syndrome**, cancer in the bone marrow resulting in low numbers of one or more type of blood cells<sup>12</sup>



**Soft tissue sarcoma**, cancer developed from soft tissue<sup>13</sup>



**Chronic lymphocytic leukemia**, a slow-growing rare disease that belongs to a family of blood cancers classified as B-cell malignancies<sup>14</sup>

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## References

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