

Off-label Use of Interferon Gamma-1b for Osteopetrosis: A Case Report



Lucy Lin

Pharmacy Department, Westmead Hospital, NSW

Acknowledgements: Dr Jennifer Gunton
Department of Endocrinology, Westmead Hospital
NSW



Health
Western Sydney
Local Health District

Introduction

Osteopetrosis is a rare genetic condition, affecting an estimated 1 in 100 000 to 500 000 people. It is characterised by increased bone mass and skeletal fragility due to defective osteoclast resorption and impaired bone remodelling. This can lead to bone marrow failure, frequent fractures and infections¹.

Objective

To describe the off-label use of interferon gamma-1b (IFN) for osteopetrosis

Clinical Features

A 52 year old female was transferred to a large teaching hospital with pain and swelling secondary to recurrent mandibular osteomyelitis. Relevant background medical history included osteopetrosis. On admission, she experienced symptoms of worsening vision, hearing impairment, anaemia and pancytopenia.

Interventions

The medical team decided to trial IFN subcutaneous injection 1.5 micrograms per kilogram of body weight per dose three times a week based on the results of limited overseas paediatric and adult trials and the severity of her vision impairment and current infection^{2,3}. It is hypothesised that IFN stimulates osteoclast function which increases the degradation of the bone matrix³. Pharmacy was involved in dose calculation and organisation of supply. Subcutaneous IFN 70 microgram (based on a weight of 48kg) three times weekly was initiated, with the first dose administered in hospital. Local drug committee approved an 8 week trial.

Conclusion

To our knowledge, this is the first reported case describing the off-label use of IFN in Australia. It may be a more cost-effective and less invasive option compared to other treatments such as bone marrow transplants^{2,3}. Long-term outcomes such as reduction in infection risks and pathological fractures will need to be explored in the future.

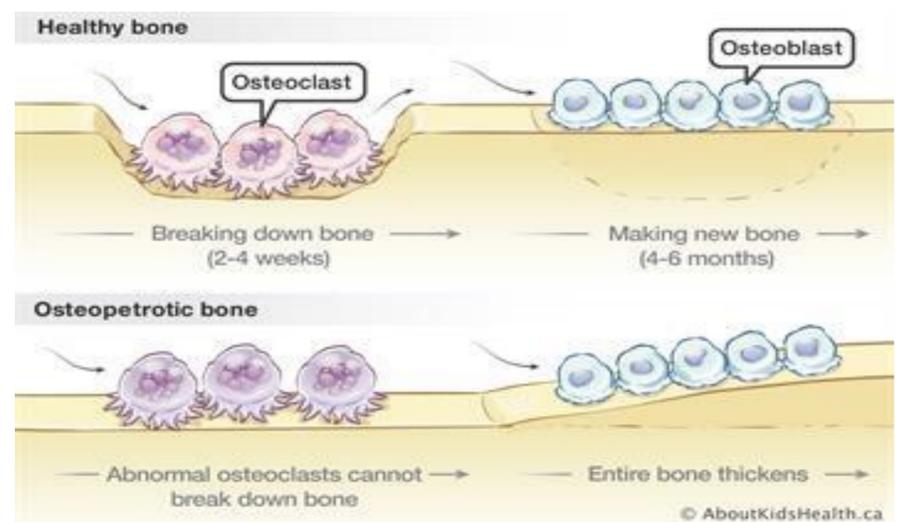


Figure 1. Healthy bone formation versus osteopetrotic bone formation

Case progress and outcomes

The patient was discharged after her first dose. Eight weeks post discharge, IFN was decreased to 50 micrograms per dose due to flu-like symptoms. Her haemoglobin had improved during this period. She has since received funding from her local hospital for continuing therapy of IFN.

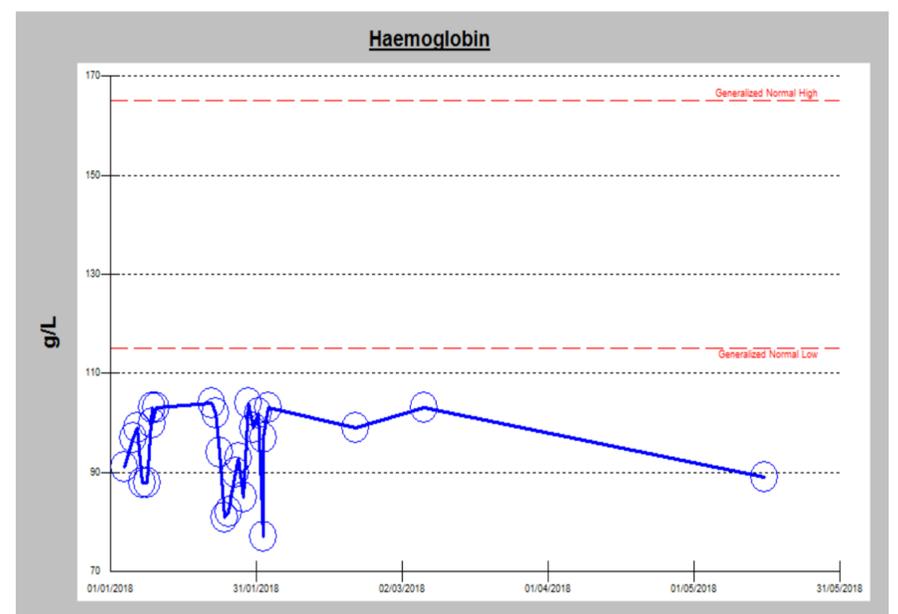


Figure 2: Haemoglobin results before and during period of treatment

References:

1. Robert B. Osteopetrosis: Background, Etiology [Internet]. Emedicine.medscape.com. 2017 [cited 7 November 2018]. Available from: <https://emedicine.medscape.com/article/123968-overview>
2. Key L, Rodriguez R, Willi S, Wright N, Hatcher H, Eyre D et al. Long-Term Treatment of Osteopetrosis with Recombinant Human Interferon Gamma. *New England Journal of Medicine*. 1995;332(24):1594-1599.
3. Key L, Ries W, Rodriguez R, Hatcher H. Recombinant human interferon gamma therapy for osteopetrosis. *The Journal of Pediatrics*. 1992;121(1):119-124.