Successful treatment of a Complex Vascular Malformation with Sirolimus and Surgical Resection

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INTRODUCTION: Large complex vascular malformations are rare condition associated to major body deformities and disfiguration, pain, recurrent bleeding, infections, high-output heart failure and, ultimately, death may occur [1]. Typically, management represents a challenge since it may include a wide variety of options such as embolization, laser therapy, sclerotherapy and surgical resection but may lead to significant morbidity [2]. In extreme situations, lesions considered otherwise untreatable have been reported to respond to a new alternative treatment: mammalian target of rapamycin inhibitors (mTORI) [1,3,4].

METHODS AND RESULTS: We report a case of giant complex vascular malformation treated with sirolimus followed by surgical resection. A 19-year old woman was referred to our department with long history of large giant soft tissue mass compromising the entire left hemithorax causing pain and crippling discomfort. Lesion was considered unresectable. CT-scan showed massive vascular slow-flow lesion that invaded deeper structures including abdominal musculature, the peritoneal cavity with extension around the anterior and posterior ribs, diaphragm and anterior mediastinum. Estimated volume was 8400 mL. Because previous treatments were ineffective, therapeutics with oral sirolimus was considered. Therapy was well tolerated. None of commonly reported side events were seen (gastrointestinal or bone marrow toxicity, rashes, mucositis, headaches) [5]. By the end of the second year into therapy, the mass volume substantially decreased in size and CT-scan demonstrated extensive shrinkage as well as a much less infiltrative pattern with surrounding structures. Decision to operate was based on the significant reduction in the volume of the mass associated with the clinical finding of increased elasticity in surrounding tissues, favoring a subtotal or near-total excision of the lesion and primary reconstruction. Six months after surgery, patient was asymptomatic and had improved quality of life.

CONCLUSION: Management of complex vascular malformation remains a challenging problem. Surgical treatment must be related to elevated recurrence rates, while sclerotherapy alone offers limited results and current medical therapy is associated with considerable side effects [2,5]. The mTORIs emerged as a promising new medical treatment option for vascular anomalies, particularly for vascular malformations. Refractory or in progression lesions are subject to additional benefit. mTORIs should be considered part of the armamentarium in the management of these conditions. Further studies are needed to better determine is effects and to compare sirolimus to newer mTORI.

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