Cost and Opportunity in Hemophilia: The Economic Impact of Drug Treatment

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Objetives

To measure the cost of prophylactic treatment and bleeding management in a 12-year-old child weighing 42kg[1] with severe hemophilia with or without inhibitors from the perspective of the Brazilian healthcare system.
For non-inhibitor treatment, we compared emicizumab vs FVIII and for inhibitor treatment, emicizumab vs bypass agents.

Methods

This study used local guidelines regimens[2,3], prices[4] and annual bleeding rates (ABR): for bypass agents (21.1), FVIII (4.8), emicizumab (0.3) for inhibitor treatment and (1.3) non-inhibitor[5-7].
For the annual treatment, we considered the weekly-recommended dose of 7020 mcg/kg for recombinant activated factor VII (rFVIIa), 78mg/kg for emicizumab, 4151U/kg for FVIII, multiplied by 52 weeks. Except for rFVIIa, which can only be used for 6 months (26 weeks) and FVIII that is based on a cohort study[8].
For the annual bleeding treatment, we considered the ABR and a mean consumption for each breakthrough bleeding was 270mgc/kg of rFVIIa to treat inhibitor patients on emicizumab or rFVIIa. For non-inhibitor patients, 46.7U/kg of FVII was considered when using FVIII and 43U/kg of FVIII when using emicizumab[9]. The unitary costs were: R$3.59/mcg for rFVIIa[4]; R$174.05/mg for emicizumab[4] and R$1.10 for FVIII[4].

Results

	Inhibitor patient		
	Profhylaxis	**Bleed treatment**	**Total annual cost**
rFVIIa	R$2,910,807.90	R$2,362,232.57	R$5,273,040.47
Emicizumab	R$1,568,016.45	R$33,586.25	R$1,601,602.70
	Non inhibitor patient		
FVIII	R$191,776.20	R$10,356.19	R$202,132.39
Emicizumab	R$527,384.55	R$28,479.53	R$555,864.08

Conclusions

Despite the more expensive unitary cost, because of the lower quantity and greater efficacy in bleeding control, emicizumab costs less to treat when compared to rFVIIa and immune tolerance, when the high-dose protocol is used.
Emicizumab represents a saving of R$3,671,437.77 when compared to rFVIIa, which can pay up to 10 non inhibitor patients to switch froFVIII to emicizumab.

Acknowledgements

Nicolas Segre for his contribution with the calculation of the cost of the treatment.

Reference

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