

**FARMACI CON EVIDENZA SCIENTIFICA A SUPPORTO DELL'USO IN PEDIATRIA
PER INDICAZIONI TERAPEUTICHE DIVERSE DA QUELLE AUTORIZZATE**

ATC = G-H (apparato genito-urinario e ormoni sessuali)

Principio attivo	Indicazione terapeutica off label	Referenze di letteratura
FINASTERIDE	Irsutismo iperandrogenico dell'adolescente	Beigi A, et al, Finasteride versus cyproterone acetate-estrogen regimens in the treatment of hirsutism. Int J Gynaecol Obstet. 2004 Oct;87(1):29-33. Lumachi F, et al. Use of cyproterone acetate, finasteride, and spironolactone to treat idiopathic hirsutism. Fertil Steril. 2003 Apr;79(4):942-6. Bayram F, et al. Comparison of high-dose finasteride (5 mg/day) versus low-dose finasteride (2.5 mg/day) in the treatment of hirsutism. Eur J Endocrinol. 2002 Oct;147(4):467-71. Numerosi studi dimostrano l'efficacia del Finasteride nel trattamento dell'irsutismo idiopatico. Non esiste tuttavia alcuno studio specificamente effettuato in pazienti adolescenti. 1: J Clin Endocrinol Metab. 1999 Apr;84(4):1304-10. A prospective randomized trial comparing low dose flutamide, finasteride, ketoconazole, and cyproterone acetate-estrogen regimens in the treatment of hirsutism. Venturoli S, Marescalchi O, Colombo FM, Macrelli S, Ravaioli B, Bagnoli A, Paradisi R, Flamigni C. 2: Fertil Steril. 1999 Jul;72(1):41-6. Comparison of finasteride and flutamide in the treatment of idiopathic hirsutism. Falsetti L, Gambera A. 3: J Clin Endocrinol Metab. 2000 Jan;85(1):89-94. Comparison of spironolactone, flutamide, and finasteride efficacy in the treatment of hirsutism: a randomized, double blind, placebo-controlled trial. Moggetti P, Tosi F, Tosti A, Negri C, Misciali C, Perrone F, Caputo M, Muggeo M, Castello R. 4: Clin Exp Obstet Gynecol. 2000;27(3-4):203-6. A comparative study of a gonadotropin-releasing hormone agonist and finasteride on idiopathic hirsutism. Bayhan G, BahÅşeci M, Demirkol T, Ertem M, Yalinkaya A, Erden AC. 5: Gynecol Endocrinol. 2003 Feb;17(1):57-63. The benefits of finasteride for hirsute women with polycystic ovary syndrome or idiopathic hirsutism. Lakryc EM, Motta EL, Soares JM Jr, Haidar MA, de Lima GR, Baracat EC. 7: Eur J Endocrinol. 2004 Mar;150(3):351-4. A comparison between spironolactone and

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		spironolactone plus finasteride in the treatment of hirsutism. Keleştimur F, Everest H, Unlühizarci K, Bayram F, Sahin Y.
IDROCORTISONE EMISUCCINATO	Iperplasia surrenalica congenita, Insufficienza adrenocorticale	66 RCT esclusivamente pediatrici, 47 RCT escludendo l'uso topico (8 RCT iperplasia surrenalica congenita, 8 RCT asma, 4 RCT insufficienza adrenocorticale in neonati pretermine)
TESTOSTERONE UNDECANOATO	Sindrome di Klinefelter* *La rimborsabilità dell'uso off-label è intesa anche per la popolazione adulta	1. Partsch C.J. et al, <i>Injectable testosterone undecanoate has more favourable pharmacokinetics and pharmacodynamics than testosterone enanthate</i>. 2. Eur J Endocrinol 1995; 132: 514-519 3. Saad F. et al, <i>More than eight years' hands-on experience with the novel long-acting parenteral testosterone undecanoate</i>. Asian J Androl 2007; 9:291-297 4. Zitzmann M. et al, IPASS: a study on the tolerability and effectiveness of injectable testosterone undecanoate for the treatment of male hypogonadism in a worldwide sample of 1,438 men. J Sex Med 2013; 10:578-588 5. Nieschlag E. et al, Current topics in testosterone replacement of hypogonadal men. Best Pract Res Clin Endocrinol Metab 2015; 29:77-90 6. Wolf J. et al, Effectiveness and tolerability of parenteral testosterone undecanoate: a post-marketing surveillance study. Aging Male 2017; 20: 225-234
TRIPTORELINA (somministrazione sottocutanea)	Diagnosi della pubertà precoce centrale nei pazienti che presentano un sospetto.	1. Freire AV, Escobar ME, Gryngarten MG, Arcari AJ, Ballerini MG, Bergadá I, Ropelato MG. High diagnostic accuracy of subcutaneous Triptorelin test compared with GnRH test for diagnosing central precocious puberty in girls. Clin Endocrinol (Oxf). 2013;78(3):398-404. 2. Poomthavorn P, Khlairit P, Mahachoklertwattana P. Subcutaneous gonadotropin-releasing hormone agonist (triptorelin) test for diagnosing precocious puberty. Horm Res. 2009;72(2):114-9.