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EXPRESSION OF HUMAN FVIII COAGULATION FACTOR IN PLANTS

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Hemophilia A is a inherited bleeding disorder caused by deficiency of coagulation factor VIII (FVIII). The FVIII replacement therapy with infusion of plasma-derived or recombinant functional FVIII protein is the current standard treatment for this disease. Transgenic plants are largely used for synthesis and accumulation of foreign proteins as they represent a cost-effective alternative to microbial systems. Our aim is to transform tobacco plants with the cDNA encoding for the B-domain-deleted FVIII (FVIII-BDD). The FVIII-BDD protein extracted from the extracellular media of human cells or Chinese hamster ovary cells exhibits coagulant activity. Because of few data are available in literature about the exact composition of the common drugs for the haemophilia A treatment, some preliminary biochemical experiments have been carried out to characterize the behavior of different FVIII products currently used for the factor replacement therapy. Our data show that different pharmaceuticals contain both the whole chain and the two polypeptides (light chain and heavy chain) generated by the intracellular FVIII proteolysis. The cloning strategy to produce the FVIII in plants requires the insertion of the human gene into a plasmid capable of expressing the gene construct in plant cells. Here we describe the FVIII cloning and the preliminary data obtained by experiments carried out on tobacco protoplasts transiently transformed with the construct encoding the FVIII-BDD protein.