

**Thiolated iminosugars as ligands for gold nanoparticles to address Gaucher disease**F. Milano¹, F. Bucu¹, C. Matassini¹, F. Clemente¹, F. Cardona¹, P. Paoli², A. Goti¹, and M. Marradi¹¹Dep. of Chemistry 'Ugo Schiff', University of Florence, via della Lastruccia 3-13, 50019 Sesto F.no (FI), Italy²Dep. of Experimental and Clinical Biomedical Sciences, University of Florence, Viale Morgagni 50, 50134 Florence, Italyfrancesca.milano@unifi.it

Iminosugars are well known inhibitors of carbohydrate-processing enzymes and, in the last years, have shown attractive potential as therapeutics towards lysosomal storage disorders (LSDs), especially in the emerging pharmacological chaperone therapy (PCT)¹. PCs are molecules able to bind and stabilize misfolded enzymes involved in these diseases, resulting in enzyme activity's enhancement. Recent studies showed that the multivalent presentation of a *N*-alkylated 3,4,5-trihydroxypiperidine iminosugar in dendrimeric scaffolds affords potent inhibitors and PCs of lysosomal enzyme GCase, the deficient enzyme involved in Gaucher LSD². The efficacy of multivalency in modulating GCase activity, prompted us to multimerize the *N*-alkylated iminosugar with a 9-carbon chain (red moiety, Figure 1) using a thiol-ending linker (blue moiety, Figure 1) onto gold nanoparticles (AuNPs) as a scaffold. *In vitro* biological assays are ongoing to test the ability of these new nanosystems to act as PCs for GCase.

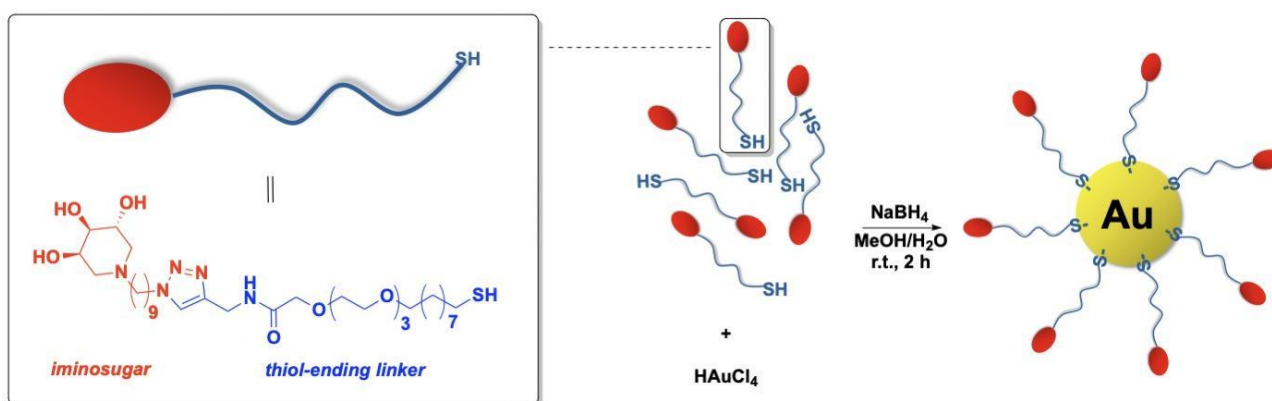


Figure 1. Multimerization of a thiol-ending *N*-alkylated iminosugar onto gold NPs by means of Au-S affinity.

References

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