FIGURE 1

HUMAN SULFATASE MODIFYING FACTOR 1 (SUMF1) POLYNUCLEOTIDE SEQUENCE (SEQ ID NO:1)

ATGGCTGCGCCCGCACTAGGGCTGGTGTGTGGACGTTGCCCTGAGCTGGGTCTCGTCCTCTTGCTGCTGCTGCTC
TCGCTGCTGTGTGGAGCGGCAGGGAGCCAGGAGGCCGGGACCGGTGCGGGCGCGGGGTCCCTTGCGGGTTCTTGC
GGCTGCGGCACGCCCCAGCGGCCTGGCGCCCATGGCAGTTCGGCAGCCGCTCACCGATACTCGCGGGAGGCTAAC
GCTCCGGGCCCCGTACCCGGAGAGCGGCAACTCGCGCACTCAAAGATGGTCCCCATCCCTGCTGGAGTATTTACA
ATGGGCACAGATGATCCTCAGATAAAGCAGGATGGGGAAGCACCTGCGAGGAGAGTTACTATTGATGCCTTTTAC
ATGGATGCCTATGAAGTCAGTAATACTGAATTTGAGAAGTTTGTGAACTCAACTGGCTATTTGACAGAGGCTGAG
AAGTTTGGCGACTCCTTTGTCTTTGAAGGCATGTTGAGTGAGCAAGTGAAGACCAATATTCAACAGGCAGTTGCA
GCTGCTCCCTGGTGGTTACCTGTGAAAGGCGCTAACTGGAGACACCCAGAAGGGCCTGACTCTACTATTCTGCAC
AGGCCGGATCATCCAGTTCTCCATGTGTCCTGGAATGATGCGGTTGCCTACTGCACTTGGGCAGGGAAGCGGCTG
CCCACGGAAGCTGAGTGGGAATACAGCTGTCGAGGAGGCCTGCATAATAGACTTTTCCCCTGGGGCAACAAACTG
CAGCCCAAAGGCCAGCATTATGCCAACATTTGGCAGGGCGAGTTTCCGGTGACCAACACTGGTGAGGATGGCTTC
CAAGGAACTGCGCCTGTTGATGCCTTCCCTCCCAATGGTTATGGCTTATACAACATAGTGGGGAACGCATGGGAA
TGGACTTCAGACTGGTGGACTGTTCATCATTCTGTTGAAGAAACGCTTAACCCAAAAGGTCCCCCTTCTGGGAAA
GACCGAGTGAAGAAAGGTGGATCCTACATGTGCCATAGGTCTTATTGTTACAGGTATCGCTGTGCTGCTCGGAGC
CAGAACACACCTGATAGCTCTGCTTCGAATCTGGGATTCCGCTGTGCAGCCGACCGCCTGCCCACCATGGACTGA

FIGURE 2

HUMAN SULFATASE MODIFYING FACTOR 1 (SUMF1) POLYPEPTIDE SEQUENCE (SEQ ID NO:2)

MAAPALGLVCGRCPELGLVLLLLLLSLLCGAAGSQEAGTGAGAGSLAGSCGCGTPQRPGAHGSSAAAHRYSREAN
APGPVPGERQLAHSKMVPIPAGVFTMGTDDPQIKQDGEAPARRVTIDAFYMDAYEVSNTEFEKFVNSTGYLTEAE
KFGDSFVFEGMLSEQVKTNIQQAVAAAPWWLPVKGANWRHPEGPDSTILHRPDHPVLHVSWNDAVAYCTWAGKRL
PTEAEWEYSCRGGLHNRLFPWGNKLQPKGQHYANIWQGEFPVTNTGEDGFQGTAPVDAFPPNGYGLYNIVGNAWE
WTSDWWTVHHSVEETLNPKGPPSGKDRVKKGGSYMCHRSYCYRYRCAARSQNTPDSSASNLGFRCAADRLPTMD

FIGURE 3

HUMAN N-ACETYLGALACTOSAMINE-6_SULFATASE (GALNS) POLYNUCLEOTIDE SEQUENCE (SEQ ID NO:3)

ATGGCGGCGGTTGTCGCGGCGACGAGGTGGTGGCAGCTGTTGCTGGTGCTCAGCGCCGCGGGGATGGGGGCCTCG
GGCGCCCCGCAGCCCCCCAACATCCTGCTCCTGCTCATGGACGACATGGGATGGGGTGACCTCGGGGTGTATGGA
GAGCCCTCCAGAGAGACCCCGAATTTGGACCGGATGGCTGCAGAAGGGCTGCTTTTCCCAAACTTCTATTCTGCC
AACCCTCTGTGCTCGCCATCGAGGGCGGCACTGCTCACAGGACGGCTACCCATCCGCAATGGCTTCTACACCACC
AACGCCCATGCCAGAAACGCCTACACACCGCAGGAGATTGTGGGCGGCATCCCAGACTCGGAGCAGCTCCTGCCG
GAGCTTCTGAAGAAGGCCGGCTACGTCAGCAAGATTGTCGGCAAGTGGCATCTGGGTCACAGGCCCCAGTTCCAC
CCCCTGAAGCACGGATTTGATGAGTGGTTTGGATCCCCCAACTGCCACTTTGGACCTTATGACAACAAGGCCAGG
CCCAACATCCCTGTGTACAGGGACTGGGAGATGGTTGGCAGATATTATGAAGAATTTCCTATTAATCTGAAGACG
GGGGAAGCCAACCTCACCCAGATCTACCTGCAGGAAGCCCTGGACTTCATTAAGAGACAGGCACGGCACCACCCC
TTTTTCCTCTACTGGGCTGTCGACGCCACGCACGCACCCGTCTATGCCTCCAAACCCTTCTTGGGCACCAGTCAG
CGAGGGCGGTATGGAGACGCCGTCCGGGAGATTGATGACAGCATTGGGAAGATACTGGAGCTCCTCCAAGACCTG
CACGTCGCGGACAACACCTTCGTCTTCTTCACGTCGGACAACGGCGCTGCCCTCATTTCCGCCCCCGAACAAGGT
GGCAGCAACGGCCCCTTTCTGTGTGGGAAGCAGACCACGTTTGAAGGAGGGATGAGGGAGCCTGCCCTCGCATGG
TGGCCAGGGCACGTCACTGCAGGCCAGGTGAGCCACCAGCTGGGCAGCATCATGGACCTCTTCACCACCAGCCTG
GCCCTTGCGGGCCTGACGCCGCCCAGCGACAGGGCCATTGATGGCCTCAACCTCCTCCCCACCCTCCTGCAGGGC
CGGCTGATGGACAGGCCTATCTTCTATTACCGTGGCGACACGCTGATGGCGGCCACCCTCGGGCAGCACAAGGCT
CACTTCTGGACCTGGACCAACTCCTGGGAGAACTTCAGACAGGGCATTGATTTCTGCCCTGGGCAGAACGTTTCA
GGGGTCACAACTCACAATCTGGAAGACCACACGAAGCTGCCCCTGATCTTCCACCTGGGACGGGACCCAGGGGAG
AGGTTCCCCCTCAGCTTTGCCAGCGCCGAGTACCAGGAGGCCCTCAGCAGGATCACCTCGGTCGTCCAGCAGCAC
CAGGAGGCCTTGGTCCCCGCGCAGCCCCAGCTCAACGTGTGCAACTGGGCGGTCATGAACTGGGCACCTCCGGGC
TGTGAAAAGTTAGGGAAGTGTCTGACACCTCCAGAATCCATTCCCAAGAAGTGCCTCTGGTCCCACTAG

FIGURE 4

HUMAN N-ACETYLGALACTOSAMINE-6-SULFATASE (GALNS) POLYPEPTIDE SEQUENCE (SEQ ID NO:4)

MAAVVAATRWWQLLLVLSAAGMGASGAPQPPNILLLLMDDMGWGDLGVYGEPSRETPNLDRMAAEGLLFPNFYSA
NPLCSPSRAALLTGRLPIRNGFYTTNAHARNAYTPQEIVGGIPDSEQLLPELLKKAGYVSKIVGKWHLGHRPQFH
PLKHGFDEWFGSPNCHFGPYDNKARPNIPVYRDWEMVGRYYEEFPINLKTGEANLTQIYLQEALDFIKRQARHHP
FFLYWAVDATHAPVYASKPFLGTSQRGRYGDAVREIDDSIGKILELLQDLHVADNTFVFFTSDNGAALISAPEQG
GSNGPFLCGKQTTFEGGMREPALAWWPGHVTAGQVSHQLGSIMDLFTTSLALAGLTPPSDRAIDGLNLLPTLLQG
RLMDRPIFYYRGDTLMAATLGQHKAHFWTWTNSWENFRQGIDFCPGQNVSGVTTHNLEDHTKLPLIFHLGRDPGE
RFPLSFASAEYQEALSRITSVVQQHQEALVPAQPQLNVCNWAVMNWAPPGCEKLGKCLTPPESIPKKCLWSH

FIGURE 5

STRUCTURE AND CHARACTERISTICS OF SECRETED HUMAN N-ACETYLGALACTOSAMINE-6-SULFATASE (GALNS) (SEQ ID NO: 5)

```
1   APQPPNILLL LMDDMGWGDL GVYGEPSRET PNLDRMAAEG LLFPNFYSAN
51  PICSPSRAAL LTGRLPIRNG FYTTNAHARN AYTPQEIVGG IPDSEQLLPE
101 LLKKAGYVSK IVGKWHLGHR PQFHPLKHGF DEWFGSPNCH FGPYDNKARP
151 NIPVYRDWEM VGRYYEEFPI NLKTGEANLT QIYLQEALDF IKRQARHHPF
201 FLYWAVDATH APVYASKPFL GTSQRGRYGD AVREIDDSIG KILELLQDLH
251 VADNTFVFFT SDNGAALISA PEQGGSNGPF LCGKQTTFEG GMREPALAWW
301 PGHVTAGQVS HQLGSIMDLF TTSLALAGLT PPSDRAIDGL NLLPTLLQGR
351 LMDRPIFYYR GDTLMAATLG QHKAHFWTWT NSWENFRQGI DFCPGQNVSG
401 VTTHNLEDHT KLPLIFHLGR DPGERFPLSF ASAEYQEALS RITSVVQQHQ
451 EALVPAQPQL NVCNWAVMNW APPGCEKLGK CLTPPESIPK KCLWSH
```

Processed form

~40 kDa

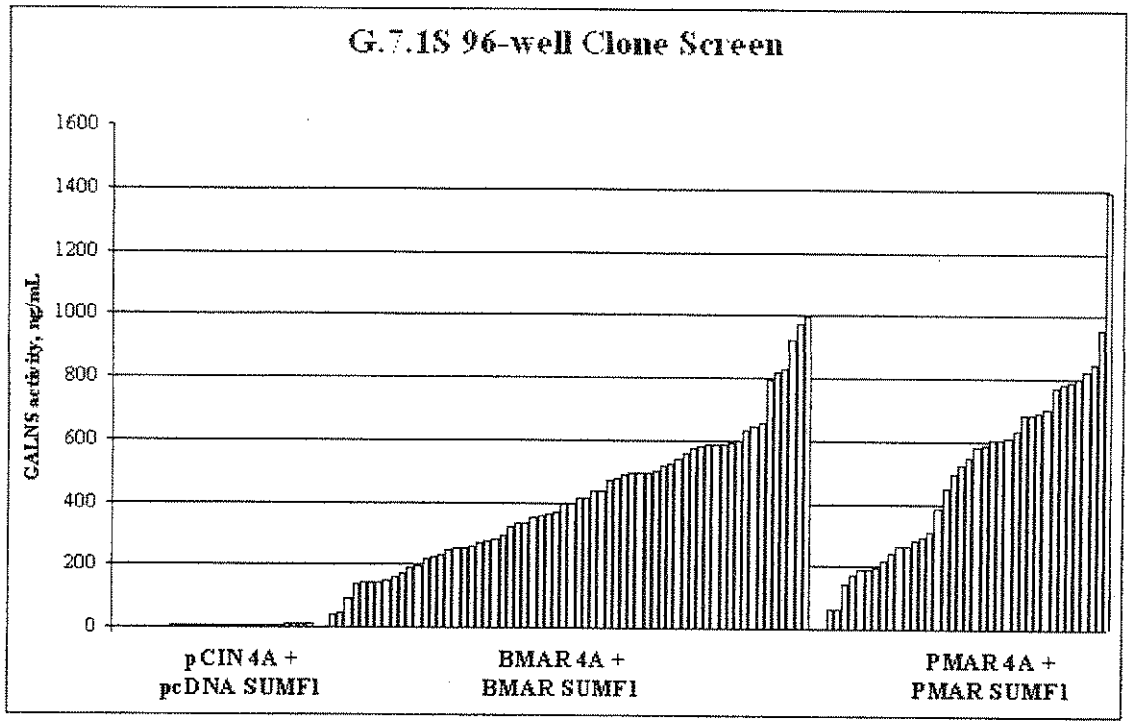
~19 kDa

1) Molecular weight of pre-processed enzyme: ~ 55 kDa
2) Cysteine to Cα–formylglycine conversion at position 53
3) Processed forms in lysosome joined by disulfide bridge
4) 2 N-linked glycosylation sites at positions 178 and 397
5) BisP found on Asn178, not on Asn397

RECTIFIED SHEET (RULE 91) ISA/EP

FIGURE 6A-B

A.



B.

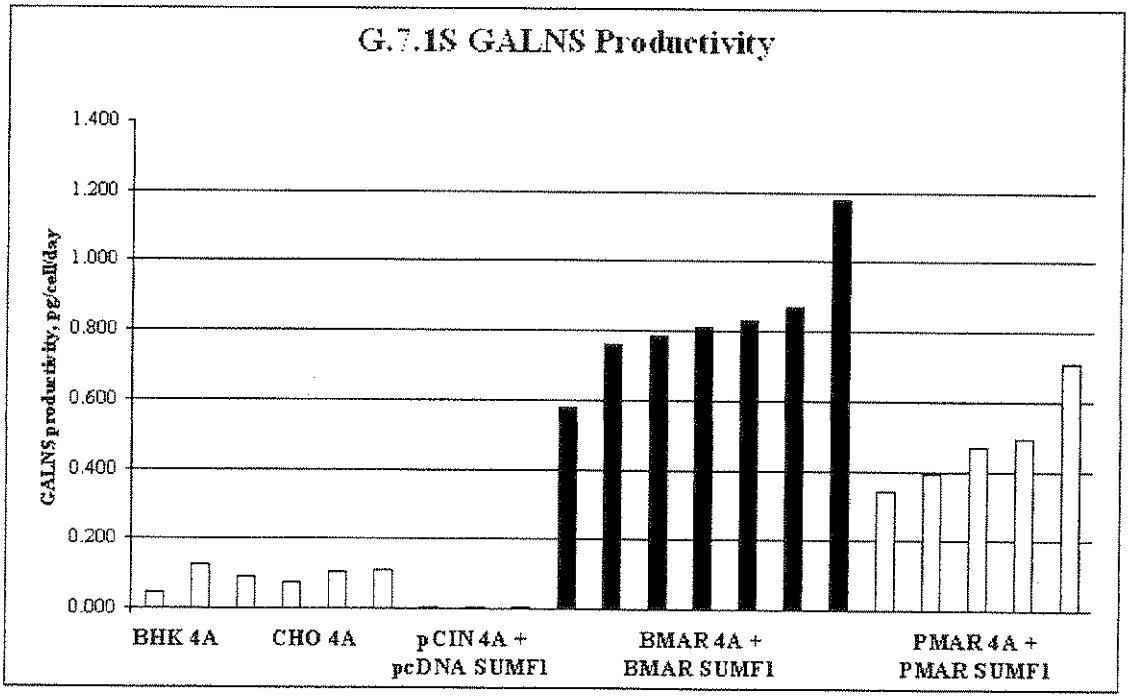


FIGURE 7

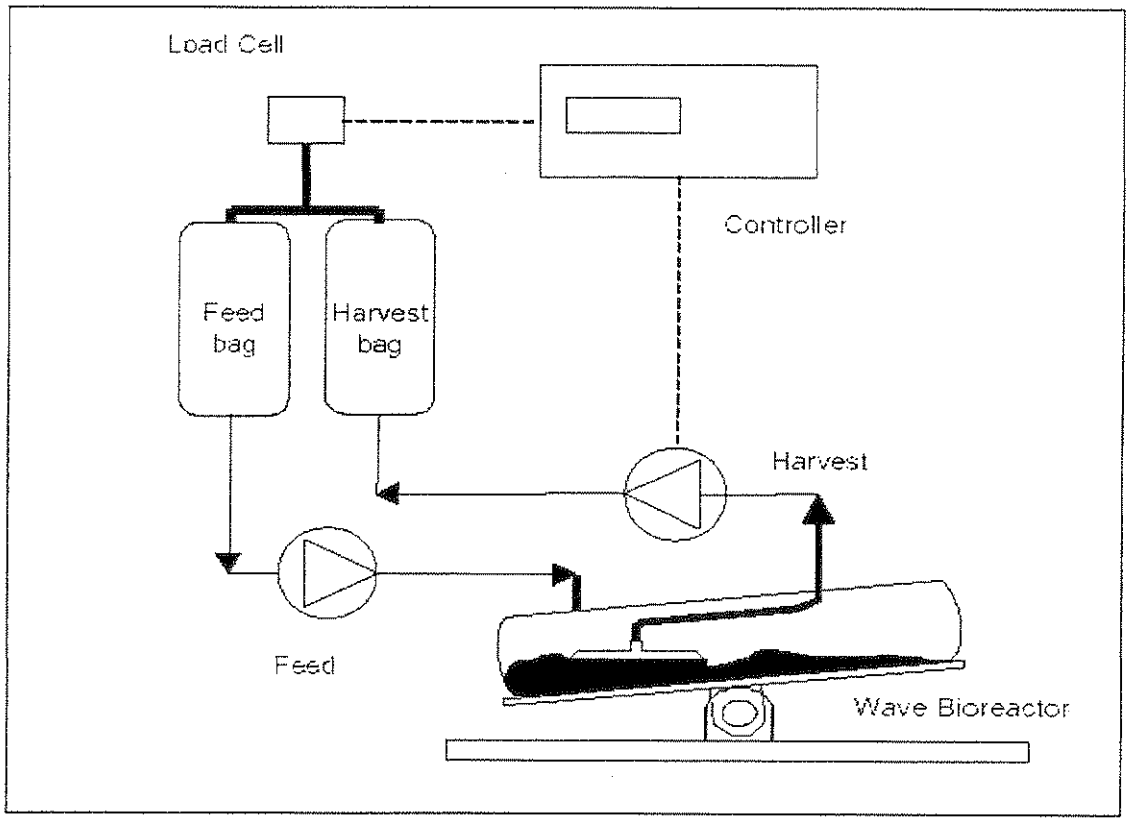

Load Cell
Controller
Feed bag
Harvest bag
Harvest
Feed
Wave Bioreactor

FIGURE 8

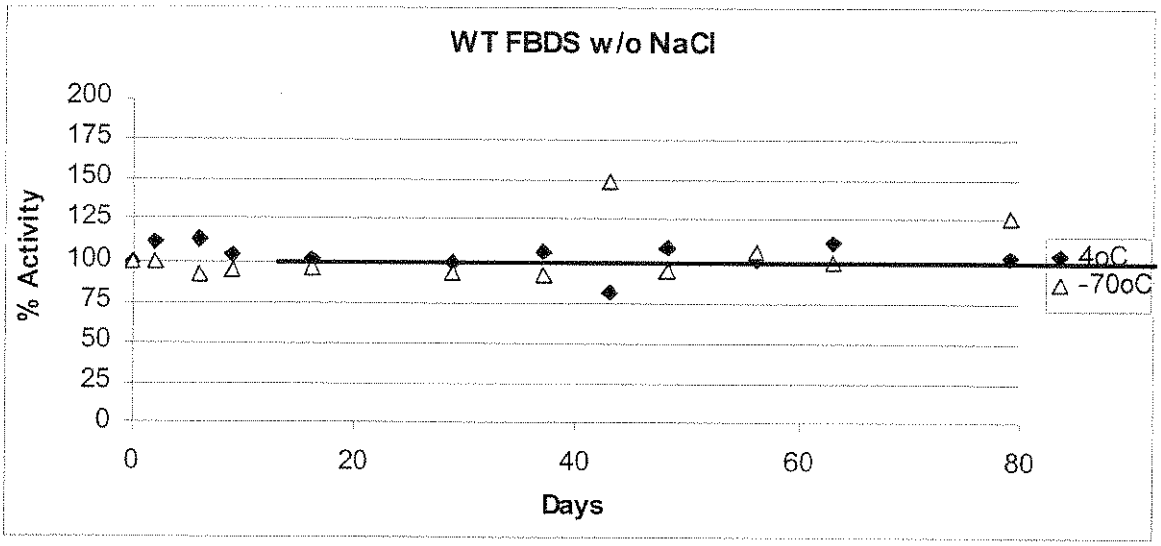
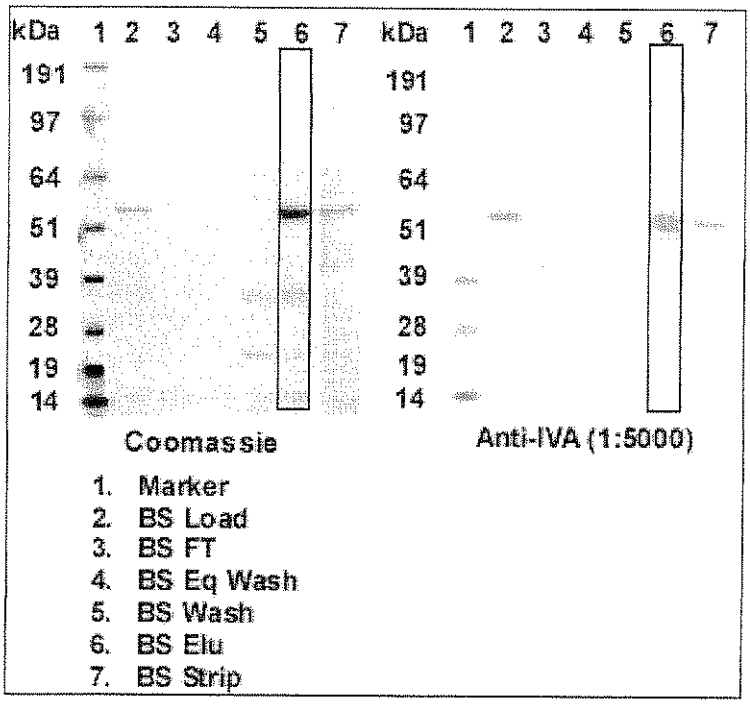

WT FBDS w/o NaCl
% Activity
200
175
150
125
100
75
50
25
0
0
20
40
60
80
Days
4oC
-70oC

FIGURE 9A-B

A. Blue Sepharose 6 FF Chromatography



B. Fractogel SE Hi-Cap Chromatography

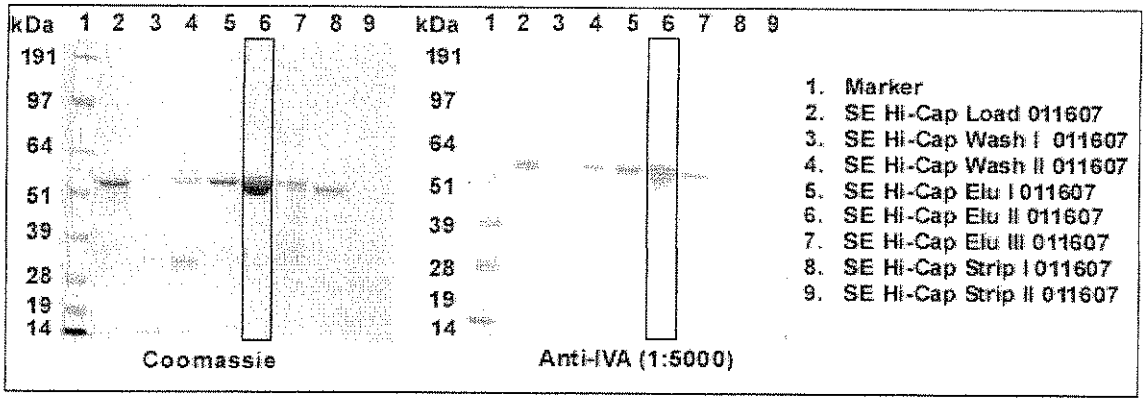
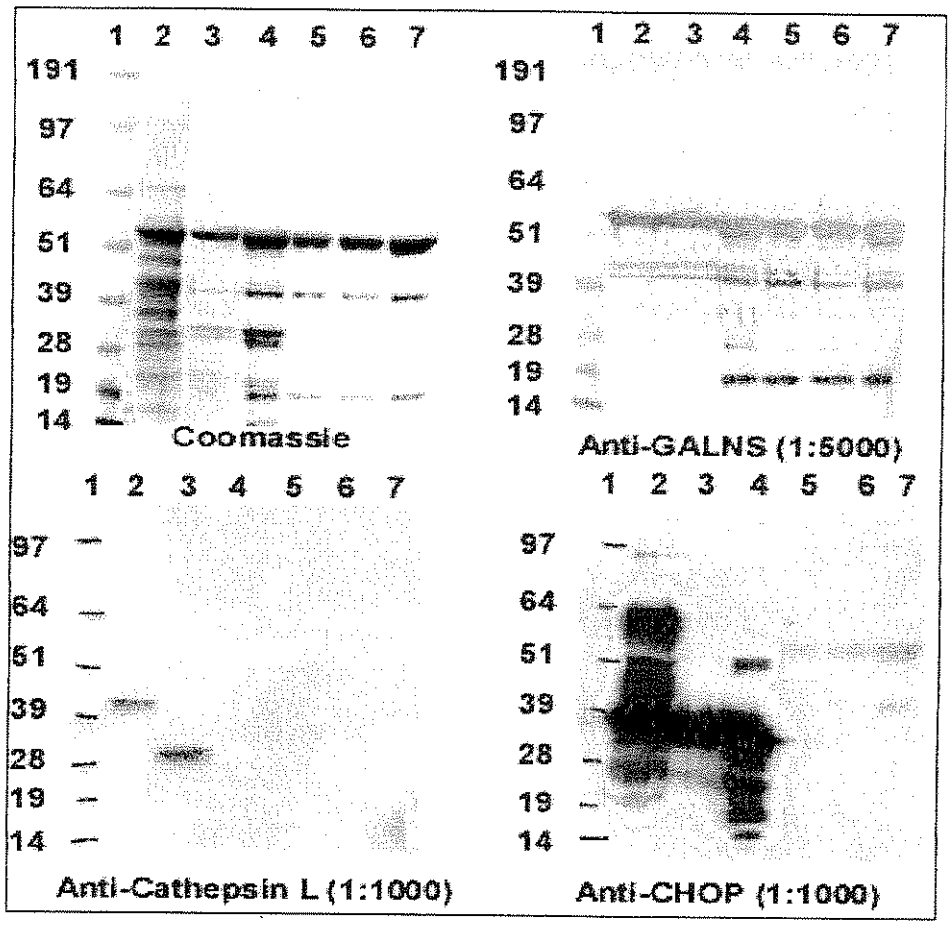


FIGURE 10



1. Marker
2. UF/DF
3. SE Hi-Cap load 042108
4. SE Hi-Cap Eluate 042108
5. Zn Eluate 042208
6. TPB Eluate 042408
7. FBDS 042508

FIGURE 11

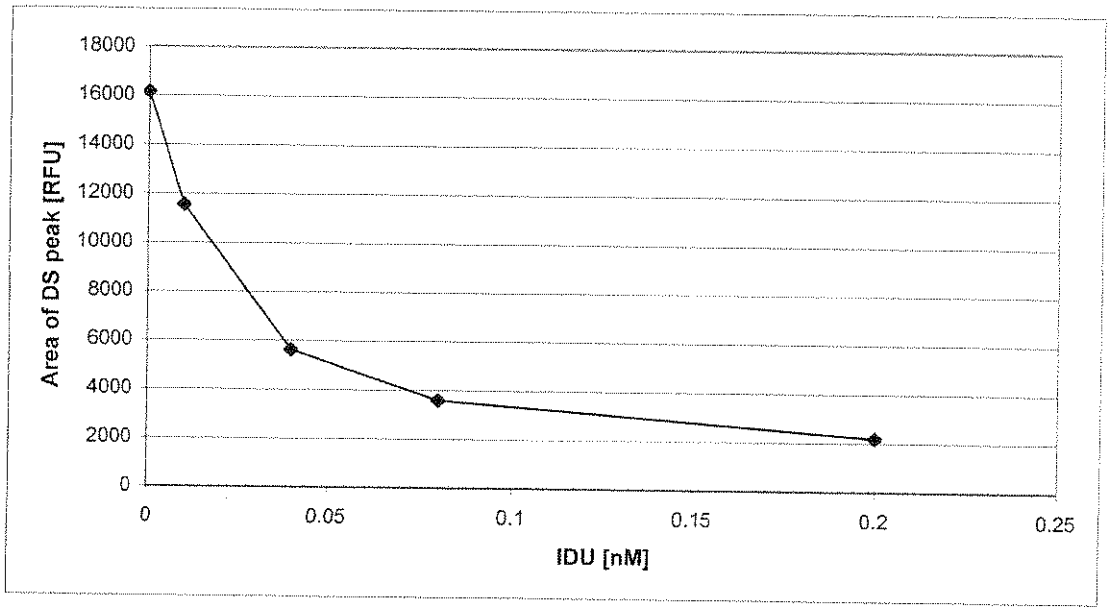

18000
16000
14000
12000
10000
8000
6000
4000
2000
0
Area of DS peak [RFU]
0
0.05
0.1
0.15
0.2
0.25
IDU [nM]

FIGURE 12

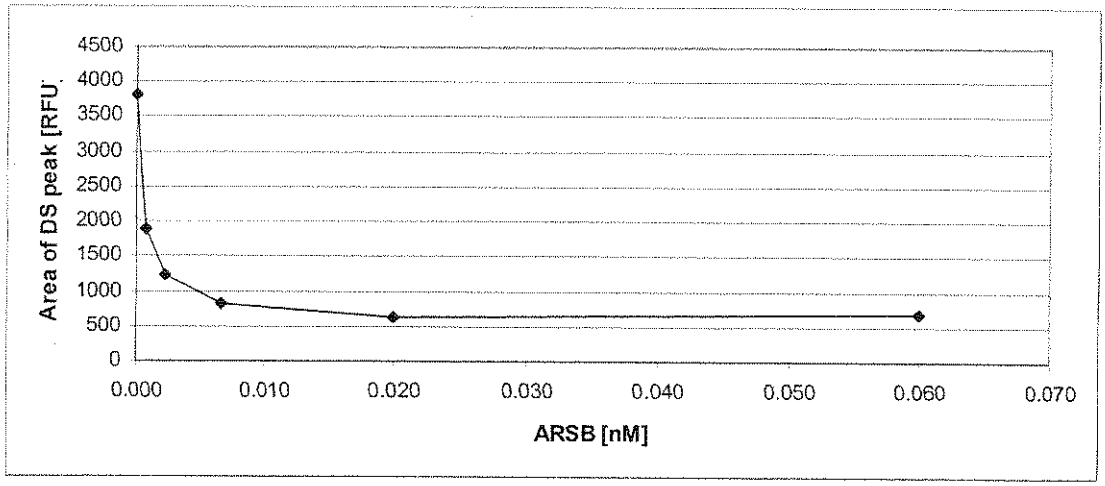
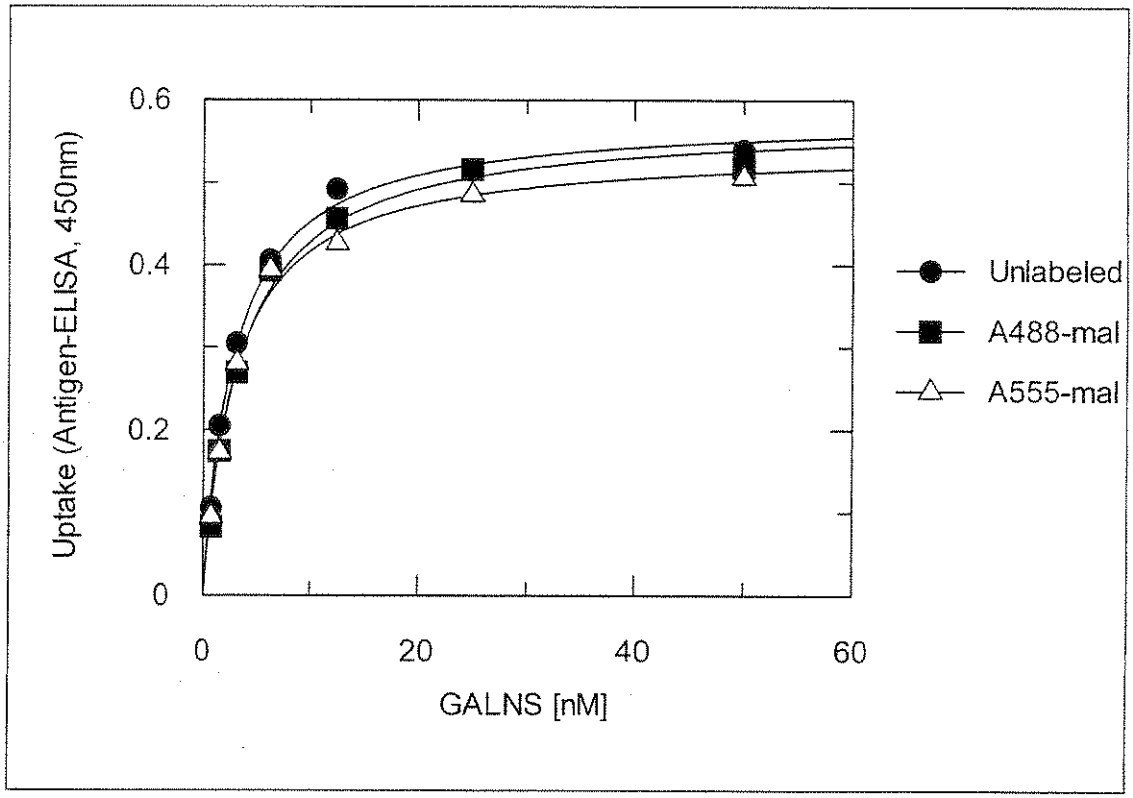

Area of DS peak [RFU]
4500
4000
3500
3000
2500
2000
1500
1000
500
0
0.000
0.010
0.020
0.030
0.040
0.050
0.060
0.070
ARSB [nM]

FIGURE 13


Uptake (Antigen-ELISA, 450nm)
0.6
0.4
0.2
0
0
20
40
60
GALNS [nM]
Unlabeled
A488-mal
A555-mal