|  |  |  |  |
| --- | --- | --- | --- |
| **Table 1** **Patient characteristic** | | | |
| Characteristic | N | % |
| **Gender** |  |  |
| Female | 112 | 67 |
| Male | 56 | 33 |
| **Primary / Recurrent** |  |  |
| Primary | 140 | 83 |
| Recurrent | 28 | 17 |
| **Mean age, years (range)** | 39.1 (1 - 88) | |
| **Mean size, mm (range)** | 74.7 (13.2 – 255.8) | |
| **Site** |  |  |
| Extremities | 50 | 30 |
| Abdominal wall | 45 | 27 |
| Other trunk | 47 | 28 |
| Retroperitoneal | 6 | 4 |
| Neck | 20 | 12 |
| ***CTNNB1* mutation (n= 154)** | | |
| T41A | 91 | 59 |
| T41I | 5 | 3 |
| S45F | 17 | 11 |
| S45P | 6 | 4 |
| H36P | 1 | 1 |
| Wild type | 34 | 22 |
| **Drug administration** | | |
| COX-2 inhibitor | 116 | 69 |
| Tranilast | 26 | 15 |
| Tamoxifen | 3 | 2 |
| None | 42 | 25 |
| **RECIST (n = 156)** | | |
| CR | 7 | 4 |
| PR | 17 | 11 |
| SD | 64 | 41 |
| PD | 68 | 44 |

CR; complete remission, PR; partial remission, SD; stable disease, PD; progressive disease

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| **Table 2 Univariate analysis of factors associated with tumor progression** | | | | | |
| Factor |  | | Non-PD | PD | p value | |
| **Gender** | | Female | 55 | 50 | 0.14 | |
|  | | Male | 33 | 18 |  | |
| **Age** | | < 33 | 31 | 32 | 0.14 | |
|  | | > 33 | 57 | 36 |  | |
| **Size** | | < 64mm | 39 | 38 | 0.15 | |
|  | | > 64mm | 49 | 30 |  | |
| **Primary / Recurrent** | | Primary | 75 | 55 | 0.47 | |
|  | | Recurrent | 13 | 13 |  | |
| **Site (Neck/Other)** | | Neck | 8 | 9 | 0.41 | |
|  | | Other | 80 | 59 |  | |
| **Site (Extremity/Other)** | | Extremity | 22 | 25 | 0.11 | |
|  | | Other | 66 | 43 |  | |
| ***CTNNB1* mutation** | | S45F | 6 | 9 | 0.19 | |
| **(n = 143)** | | Other | 74 | 54 |  | |
| **Drug administration** | | Presence | 63 | 54 | 0.26 | |
|  | | Absence | 25 | 14 |  | |
| **Conversion to AT** | | Presence | 20 | 47 | < 0.0001 | |
|  | | Absence | 68 | 21 |  | |

AT; active treatment

|  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | **Table 3 Univariate and multivariate analysis of factors associated with event-free survival** | | | | | | | |  | |
| Factor | |  | n | Events | Univariate HR  (95% CI) | Univariate p value | Multivariate HR  (95% CI) | Multivariate p value | |
| **Gender** | |  |  |  |  |  |  |  | |
|  | | Female | 113 | 53 | 1.3 (0.8 – 2.2) | 0.29 | NA |  | |
|  | | Male | 55 | 21 | Ref |  |  | |
| **Age** | |  |  |  |  |  |  |  | |
|  | | < 33 | 93 | 39 | 1.3 (0.8– 2.0) | 0.27 | 1.13 (0.69 – 1.83) | 0.63 | |
|  | | > 33 | 75 | 34 | Ref |  | Ref |  | |
| **Size** | |  |  |  |  |  |  |  | |
| **(n = 162)** | | < 64mm | 79 | 32 | Ref | 0.50 | Ref | 0.26 | |
|  | | > 64mm | 83 | 39 | 1.2 (0.8 -2.0) |  | 1.29 (0.81 – 2.16) |  | |
| **Primary / Recurrent** | |  |  |  |  |  |  |  | |
|  | | Primary | 141 | 60 | Ref | 0.93 | NA |  | |
|  | | Recurrent | 27 | 14 | 0.98 (0.6 – 1.8) |  |  |  | |
| **Site (Neck/Other)** | |  |  |  |  |  |  |  | |
|  | | Neck | 20 | 12 | 1.9 (1.0 – 3.5) | 0.042 | 1.80 (0.86 – 3.75) | 0.12 | |
|  | | Other | 148 | 62 | Ref |  | Ref |  | |
| **Site (Extremity/Other)** | |  |  |  |  |  |  |  | |
|  | | Extremity | 50 | 21 | 0.80 (0.5 – 1.3) | 0.38 | NA |  | |
|  | | Other | 118 | 53 | Ref |  |  |  | |
| ***CTNNB1* mutation** | |  |  |  |  |  |  |  | |
| **(n = 154)** | | S45F | 17 | 13 | 2.4 (1.3 – 4.5) | 0.003 | 1.96 (1.01 – 3.80) | 0.048 | |
|  | | Other | 137 | 59 | Ref |  | Ref |  | |
| **Drug administration** | |  |  |  |  |  |  |  | |
|  | | Presence | 124 | 54 | 0.8 (0.5 – 1.3) | 0.33 | NA |  | |
|  | | Absence | 44 | 19 | Ref |  |  |  | |
|  | |  |  |  |  | NA: Not assesed | |  | |

|  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- |
| **Table 4 Active treatment modality and tumor site** | | | | |  |  |  |
|  | **Tumor site** | | | | | | p value |
|  | **EX** | **AW** | **OT** | **RP** | | **Neck** |
| **Treatment modality** | |  |  |  | |  | 0.05 |
| MTX + VBL (n = 46) | 14 | 8 | 12 | 2 | | 10 |  |
| Pazopanib (n = 3) | 1 | 0 | 1 | 0 | | 1 |  |
| Surgery (n = 24) | 5 | 14 | 5 | 0 | | 0 |  |
| Oral MTX (n =1) | 1 | 0 | 0 | 0 | | 0 |  |

MTX; Methotrexate, VBL; Vinblastine, EX; Extremity, AW; Abdominal wall,

OT; Other trunk, RP; Retroperitoneal.

**Table 5 Studies about active surveillance in desmoid-type fibromatosis**

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Reference | N | Tumor site | PD | Shift to AT | Risk factor |
| Fiore, 200921 | 83 | Extremity 27  Trunk 5  Head and neck 3  Abdominal wall 33  Thoracic wall 9  Intra-abdominal 6 | 29  (35%) | 23  (28%) | NS |
| Bonvalot, 201320 | 102 | Abdominal wall 102 | NS | 37  (36%) | Size > 3.5cm |
| Colombo, 201522 | 70 | Intra-abdominal 10  Exttremity/girdle 26  Head/neck 2  Trunk 32 | 28  (40%) | 28  (40%) | NS |
| Burtenshaw, 201627 | 120 | Abdominal wall 63  Intra-abdominal 49  Both 8 (all FAP patients) | NS | 53  (44%) | NS |
| Penel, 201723 | 388 | NS | 117  (30%) | 71  (18%) | NS |
| Van Broekhoven, 201825 | 37 | Head/neck 3  Thorax/back 13  Abdominal wall 13  Extremity 4 | 5  (14%) | 15  (41%) | NS |
| Turner, 201928 | 50 | Head/neck 3  Upper extremity 4  Lower Extremity 14  Abdominal wall 14  Intra-abdominal 3  Chest wall 6  Breast 2  Limb girdle 2  Other 2 | 21  (42%) | 19  (38%) | NS |

**Table 5 (Continued)**

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Reference | N | Tumor site | PD | Shift to AT | Risk factor |
| Van Houdt, 201926 | 168 | Lower extremity 20  Upper extremity 31  Abdominal wall 61  Intra-abdominal 15  Chest wall 30  Other 11 | 60  (36%) | 78  (46%) | Size > 7cm  Pain  RECIST PD |
| Cassidy, 202024 | 72 | Abdominal wall 19  Chest wall 5  Intra-abdominal 21  Extremity 21  Other 6 | NS | 42  (58%) | Size > 5cm  Paraspinal/frank location |
| Cuomo, 202130 | 70 | Upper extremity 20  Lower extremity 22  Pelvic gridle 26  Shoulder gridle 31 | 38  (54%) | NS | Pain  Shoulder location |
| Soboczuk, 202129 | 139 | Abdominal wall 46  Chest wall 40  Upper extremity 10  Lower extremity 25  Intra-abdominal 15 | 60  (43%) | 41  (29%) | NS |
| This study | 168 | Neck 20  Extremity 50  Abdominal wall 45  Other trunk 47  Retroperitoneal 6 | 71/156  (46%) | 74/168  (44%) | Neck location  *CTNNB1* S45F  RECIST PD |
| AT; active treatment, PD; progressive disease, FAP; Familial Adenomatous Polyposis, NS; not specified | | | | | |